



44 Transcripts: Economists Who Answered the Questionnaire on the Pre-Market Approval of Drugs and Devices

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ABSTRACT

The present compendium of transcripts constitutes the appendix to the report on the response to the interactive questionnaire on the pre-market approval of drugs and devices.

Handy Links:

- [The January 2010 call to 305 economists](#)
- The questionnaire is in two forms: [HTML](#) (interactive), [PDF](#) (noninteractive)
- [The May 2010 report on the 44 responses compiled in the present document](#)
- [The present transcripts in spreadsheet form](#)

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Acknowledgments: We are grateful to the 44 individuals, identified in the present article, who answered our call. Also, we thank Joel Friedman and SurveyWriter, Inc. for their help in hosting the survey, and Mike Watkins for help with outputting the data to manageable forms.

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Kenneth Arrow

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Stanford University, Professor of Economics and of Management Science and Engineering Emeritus

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

(I prefer not to respond.)

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

The firms do not have the right incentives to assure efficacy and safety.

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Information is difficult to acquire and understand. The average consumer is in no position to find the information for him- or herself and will therefore be likely err relative to the full information available.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

There would be no publicly available evidence on the efficacy and safety of the drugs, except what is supplied by interested parties.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Avoidable imperfect information may inhibit the entry of rivals.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

Additional information through the NIH or (preferably) a dedicated information-disseminating information (like NICE in the U.K.) would be valuable, particularly in acquiring and diffusing information derived from post-approval use. It would not be a substitute for the controlled tests which are used in the approval process.

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

This is a closer call than the abolition of the approval process. Nevertheless, the dissemination of the relevant information is too costly for the patients (and the doctors) to permit the sale of drugs that have not met the appropriate standards. (There are many parallels in other fields.)

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

My correct answer, not provided for in your choices, is, I cannot be sure. It will certainly depend on the degree of coordination among the different countries. An international approval body makes sense; an unrestricted reliance on the agencies of other countries would require at least a study of the quality of their performance.

31: *Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?*

No

32: *Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?*

Yes

33: *What evidence can you cite for systematic failure in assuring efficacy prior to 1962?*

At the time, there were certainly some good examples. I haven't looked at this historical evidence since.

34: *Do you have any general comments about this questionnaire or further thoughts about the matters treated?*

There is a broad principle of economies of scale in the collection of information which is applicable, for example, to national income data. I cannot imagine a substitute in the case of testing drugs for both safety and efficacy. The only possible controversy is whether the government or the patient or the physician should make the final decision on the use of the information.

Pedro Pita Barros

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Universidade Nova de Lisboa

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Patients overestimate benefits and patients with insurance do not take into account the opportunity cost of their consumption.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Doctors tend to adopt too early and too fast, as signal of their quality, and as they bear no financial cost of decisions (and not do the patients if insured)

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Knowledge about the new pharmaceutical products is a public good, and a liberalized market does not have the right provision incentives.

13b: Do you believe that uncertainty per se constitutes a market failure?

Yes

14b: How in your view does uncertainty per se constitute a market failure?

Obtaining knowledge to reduce uncertainty has aspects of a public good.

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Companies have more information about the products and may use it strategically.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

I see the pre-market approval as minimum quality standard that needs to be enforced. This requires information and enforcement power. The subsidization of the generation of knowledge provides the information but not the enforcement of minimum quality standards.

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: *As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?*

No

23b: *Why not?*

The extra information brought in the process of approval is also relevant.

29b: *As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?*

Yes

31: *Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?*

No

32: *Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?*

No

34: *Do you have any general comments about this questionnaire or further thoughts about the matters treated?*

No opinion about pre-1962 efficacy, I never studied that particular issue. Bear in mind I am an European talking about the US system.

Marc L. Berger

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Eli Lilly and Company

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing

costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Patients have difficulty putting risks and harms into appropriate context. Moreover, how they put them into context is shaped by how the data is presented (ex. relative risk, absolute risk, how risk is placed into context). Moreover, the public does not fully grasp that the risk benefit profile for any treatment is always conditional -- we will always be learning more that will revise this assessment as more experience is gained with a treatment. Sometimes we will learn of additional benefits. Sometimes we will learn of additional harms. The dynamic nature of this for medical treatments is greater than that for other consumer goods.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

The government has a legitimate role in ensuring that the information disseminated to physicians and patients is accurate and fair. The evidence for benefits and harms is incomplete at the time of product launch and is therefore open to interpretation. The FDA acts as the advocate for the public to ensure a balanced interpretation of the available information.

13b: Do you believe that uncertainty per se constitutes a market failure?

Yes

14b: How in your view does uncertainty per se constitute a market failure?

See my previous comments.

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

No.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

The government has a legitimate interest in the public health and safety. If a new product is less effective and is associated with more harms than available treatments, the government appropriately should not allow it to be marketed.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Since we will always be dealing with uncertainty, then the values of different societies will impact approval decisions -- that is, different countries may draw different conclusions about what is an appropriate risk-benefit profile. What is also an acceptable profile depends on the context in which a new drug will be used. Treatment patterns differ significantly across countries. Therefore the context for approval differs.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

This was a forced choice question. Yes or no. I'm not thoroughly familiar with what happened before 1962. For one thing, there were a lot fewer prescription drugs on the market. The Thalidomide story was an important stimulus for more stringent requirements regarding testing prior to approval.

Cornelis Boersma

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

MSc (Pharm), PhD; 1) University of Groningen, Department of Pharmacy, Unit of PharmacoEpidemiology & PharmacoEconomics (PE2); and 2) Health Economics Consultancy & Technology Assessments (HECTA)

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...neither understated nor overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

e.g. gives guidance in comparing treatment options (e.g. less off-label use expected)

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Several, but among others patients' drug persistence in general

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Not only the evidence and importance of health gains but also financial aspects (e.g. profits next to drug prices) are weighted in their choices. Not always evidence based (e.g. strictly following market approval vs. off-label). Among other points, routine decisions on drugs and devices in stead of (guideline) recommended and/or evidence-based. This can lead to 'trial-and-error' treatment strategies

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: *Why not?*

The drug and medical device market should definitely be regulated to a certain extent and is therefore not suitable for liberalized system. This is due to different supply/demand framework for health care versus other product markets. In my opinion, a combined regulation/liberalization system should be preferred. Pre-market approval should go hand in hand with liberal policy after approval to also find evidence for more heterogeneous populations (other than populations in randomized clinical trials). Of course, the latter should be evidence dependent and requires (strict) monitoring to evaluate 'real-life'(cost-)effectiveness and safety leading to evidence-based medicine decisions.

13b: *Do you believe that uncertainty per se constitutes a market failure?*

No

16b: *In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.*

Clinical trial outcomes are limited to specific/selected patient populations. Therefore, interpretation and translation of outcomes ((cost-)effectiveness & safety) to 'real-life' practice is often difficult and not (always) evidence based. Valuation of existing information/evidence gives the opportunity to make health care decisions with considering existing uncertainty.

18b: *You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?*

Yes

20b: *Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?*

Yes

22b: *As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?*

No

23b: *Why not?*

Reporting on drugs and devices from a FDA perspective provides us with an independent and transparent view on (not) approved products. In case of automatic approval, it would be necessary to introduce the concept of 'risk-sharing' to make manufacturers (rather than government) responsible for the market behaviour and consequences in terms of (cost-)effectiveness, safety profile and budget impact related to health care professionals' choices on (new) drugs and devices.

24b: *You indicated that a superior ability of government to assure the safety and efficacy of pharmaceuticals justifies the policy requiring pre-market approval. Does that superiority stem from the FDA having special expertise in evaluating safety and efficacy?*

Yes

25b: *Why is it that doctors and consumers have inferior judgment in evaluating safety and efficacy?*

May be not inferior but more individualized judgement/evaluation of safety and efficacy, rather than based on a variety of expertise within FDA expert panels.

26b: *Would you say that impartiality or commitment to the public good are sources of the government's superior ability to assure safety and efficacy?*

Yes

27b: *In what ways does the FDA's impartiality or commitment to the public good render it superior to doctors and patients in judging safety and efficacy?*

- Wide variety of expertise - Independent view - No (direct) financial interest of experts - Critical and transparent - Etc

29b: *As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?*

Yes

31: *Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?*

No

32: *Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?*

No

34: *Do you have any general comments about this questionnaire or further thoughts about the matters treated?*

Regulatory systems - such as FDA pre-market approval - for drugs and medical devices based on efficacy & safety are required to judge these products, provide information and translate existing outcomes evidence to guide health care professionals. Uncertainty issues will always exist due to differences between controlled settings (selected populations) and 'real-life' settings (heterogenous populations. Several methods exist to show or value uncertainty around (cost-)effectiveness/safety outcomes. Therefore, these methods can contribute to decision-making processes (e.g. to decide on timing of a decision (here, approval) or to decide on additional requirements for additional research to reduce uncertainty). Also, follow-up monitoring of 'real-life' (cost-)effectiveness and safety profiles should be recommended to give guidance in (evidence-based) treatment choices.

John E. Brazier

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor of Health Econmics, Health Economics and Decision Science,
School of Health and Related Research

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...typically understated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

FDA is to my (British) is not Government per se, but Scientifically informed agency charged with making decisions in the public interest. In this regard I think they are better than a general public who are at the mercy of well financed advertising campaigns. Establishing that a drug is effective and safe is not something most of us can do in our spare time.

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

This is a strange question, because I am not sure what is intended by the term systematic. The problem consumers face is how to make the right decisions, when their very life may depend on getting it right - in the face of often extremely complex evidence. There might be a tendency for consumers to believe a new treatment is better, when indeed it may not be and actually do them more harm than good compared to the alternatives. Furthermore, they are usually spending the health care dollars of an insurance company or the public purse, and so this waste also has an impact on others.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Again, I do not like the term of systematic. As before, there are arguments about understanding complex scientific data and the impact on others.

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

See previous arguments about lack of information, uncertainty and externalities.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

I would add lack of information and assessment of the quality of evidence. A key role of FDA and similar agencies around the world is to provide an independent and scientifically informed review of the evidence. So it is about understanding 1) what is the likely mean effect and 2) what is the uncertainty around this. These both require someone able to understand the evidence that was collected and how it was analysed. The other issues concerns that fact that most health care is funded by a third party who need

to be assured that the scientific evidence has been properly assessed. Not something most consumers, physicians or even economists are in a position to do. However, also believe that consumers and producers should be involved in the process, particularly where there are important trade-offs to be made between different outcomes of treatment.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

There needs to be some kind of overall collation of evidence and clear guidance. Who is going to be able to read the NIH report and match this up with advertising from drug companies and really make sense of the value of the new product?

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Ditto previous arguments

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

None - this might need a review! It also has to be said that much has changed since then.

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

I hope it encourages more debate and that it is opened up to more than just economists. Most of the knowledge about this areas lies outside of economics.

James F. Burgess Jr.

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Senior Investigator, VA Center for Organization, Leadership and Management Research and Associate Professor, Boston University School of Public Health

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...neither understated nor overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Uncertainties are complex and interrelated and it is very difficult for patients (even ones who consider uncertainties well) to decompose them and think about correlations between them.

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

9a: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Physicians tend to put greater weight on the experience of their own patients and their own original training, and as such tend to resist new techniques, treatments, and procedures more than more objective evidence might support.

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

11a: Why not?

As stated, while patients have information problems and inefficiencies, so do providers, so providers in general would not be superior in this liberalized system.

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

16a: Why not?

Why do the testing under the auspices of the FDA if it is not to have implications, uncertainties in these processes are inherent and complex (using the Dave Snowden sense of complex so that it also is inherently resistant to modeling to simplify).

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

Yes

18a: How would you describe or explain the government's superior ability to judge safety and efficacy?

I really did not like the choices of yes and no and the previous question, since neither one seems to be correct or represents my opinion. Government is possibly capable of establishing this superior ability, relative to consumers the more that the consumer is not repeatedly exposed to the choices and treatments, and relative to providers the more that the alternative choices affect the providers' income and professional standing; and to the degree that government can evade commercial and regulatory capture.

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

20a: Why not?

Again, yes and no do not properly reflect a choice I can make categorically. A major problem in the pharmaceutical industry that the market is worldwide, but regulation is done by country, in that sense related approval in other

countries applying in the US could be more efficient; however, local contextual factors in each country also should be brought to bear and could not be with automatic cross country approval.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

I found the attempt to force choices to Yes/No felt artificial, and do not match the sensemaking and community contextual views I try to apply to these sorts of questions. I thought the last questions on 1962 were more or less irrelevant since the science and marketing of pharmaceuticals and the process for evaluating them has changed so much since then. Efficacy studies need to be conducted with a better sense of context so that they reflect real world treatment conditions better though.

Noel D. Campbell

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Assoc. Prof. of Econ., University of Central Arkansas

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...typically overstated.

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...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

9a: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Conservatism in treatment, and redundant testing and treatment regimes; primarily to protect against potential malpractice claims.

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

J. Jaime Caro

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor McGill University; Sr VP UnitedBiosource Corp.

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...typically understated.

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Oppose, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

overconfidence in the regulatory process dismissing of risk misinterpretation of probabilities

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

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12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

16a: Why not?

Because the consumers - doctors and patients - cannot properly evaluate the data

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

Yes

18a: How would you describe or explain the government's superior ability to judge safety and efficacy?

call on experts who know what to do time and funding

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

systematic is hard to support more of a subjective sense

William S. Comanor

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

UCLA and UCSB

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

(I prefer not to respond.)

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

(I prefer not to respond.)

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

Anthony John Culyer

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor of Economics, University of York England, and Ontario Research
Chair in Health Policy and System Design, University of Toronto, Canada

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

(I prefer not to respond.)

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

(I prefer not to respond.)

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

Commercial pressures for too-early marketing (e.g. thalidomide); complexity of appropriate decisions about prescribing; misleading marketing; contradictory clinical guidelines; direct to consumer advertising; weak and variable agency relationships,...

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Most doctors not qualified to evaluate evidence of efficacy or risk of harm. most doctors have irrational attitudes to risk. I prefer evidence-informed prescribing.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

15b: If neither consumers nor doctors err systematically in these matters and uncertainty per se does not constitute a market failure, then in what sense do you believe that imperfect information is a source of the market failure that justifies the policy requiring pre-market approval?

There can be lots of error without it being systematic or tending in a singular direction. Plain ignorance, which in some liberal worlds would be rampant, is likely to do indiscriminant harm as well as occasional good.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

No

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Many rational insurers (like mine) would want evidence of cost-effectiveness in order to offer competitive premiums for insurance against evidence-informed prescriptions.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Generalization from one jurisdiction/culture/etc. is hazardous. That's not to say there could not be useful learning.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

You gave me no choice. The Q'aire is biased. My truthful answer is that I do not know what the situation was then.

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

The questions are too framed.

Thomas DeLeire

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Associate Professor, University of Wisconsin-Madison

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Consumers are often over-sensitive to small risks. Consumers, as a result, would tend to avoid untested drugs when they are brought to market. Companies will have a private incentive to test their products in a transparent and fair manner, even without mandated FDA approval.

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Your survey seems to be biased in its design towards a finding that the current system should be liberalized. Responses suggesting that the current

system be liberalized did not require any justification or further elaboration. Responses suggesting the current system should be kept in place did require written justification. It is likely clear to respondents, as a result, which answers the survey designers were looking for and would agree with. I suggest that future iterations of this survey request justification and elaboration for each answer.

David Dranove

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor of Health Industry Management, Kellogg School of Management,
Northwestern University

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...often overstated.

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

13b: Do you believe that uncertainty per se constitutes a market failure?

No

15b: If neither consumers nor doctors err systematically in these matters and uncertainty per se does not constitute a market failure, then in what sense do you believe that imperfect information is a source of the market failure that justifies the policy requiring pre-market approval?

It is a source of market failure when other mechanisms for informing consumers prove costly, inadequate, or create perverse incentives. In the case of medications, information is lacking among buyers and sellers alike, and systematic trials (along with the accompanying publicity) may be the best way to develop and organize information so that doctors and patients can understand the benefits/drawbacks of new drugs.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Perhaps this is a short term concern, but the FDA testing process is viewed as an imprimatur of quality. This is one of the great success stories of the 1962 Amendments. I fear that companies will market drugs as having completed the testing process and consumers will draw inappropriate inferences.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

Perhaps I am more concerned about safety than efficacy in discussing the 1962 Amendments. My own research on the FDA Amendments documented three recently approved drugs that had dangerous side effects as well as Thalidomide, whose approval in the U.S. was stalled almost by accident (thanks to the efforts of a lone staffer.)

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

I am much less concerned about asymmetric information than I am about the public good nature of research. Systematic clinical trials will facilitate learning by physicians. It is not at all obvious whether a manufacturer will wish to promote learning in a way that maximizes social welfare. I would like to see widespread availability of drugs that are in what we currently call Phase III of the development process (after proving safety and potential efficacy) in Phases I and II, but I think this would require coordination among prescribing physicians so that information about patient outcomes could be aggregated. Perhaps we could designate medical centers as advanced centers for experimental treatments and they could have full access to any drugs that make it past phases I and II. This would knock 2-3 years off the approval process.

Randall P. Ellis

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Boston University, Department of Economics, Professor

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

Insurance removes the effectiveness of the market in distinguishing socially beneficial drugs from those that are not worth the cost or risk.

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Behavior economics and studies of risk aversion show that consumers have a hard time making intelligent decisions when confronted with risky prospects or decisions over time. Cutler, Finkelstein and McGarry AER 2008 is one good example.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Doctors err in prescribing in the same way that the entire medical system does because of the effects of insurance. The costs of pharmaceuticals is largely irrelevant to their decisions, only whether there is a positive benefit. Direct to consumer advertising of pharmaceuticals is also not particularly informative.

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending

a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

For the reasons previously mentioned. Too many suspect new drugs would be prescribed.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Agency problems in getting doctors to have the correct incentives for treatment.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

No

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Consumers do not pay the full cost of the drugs they use. Insurers and the government do. Hence it is appropriate for the government to play a role in deciding what they are willing to pay for.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

If the US adopted this policy, then pharma companies would just try to get approval of a new drug in the country where the review policies were the most lax. I would approve of allowing other country's information to accelerate or otherwise affect the US review process.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

The questionnaire did not permit the respondent to show the degree of certainty to most of the answers, particularly the last question. I have no particular knowledge about drug review policies before 1962, so it is unfair to force an answer to that question.

Denis Getsios

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

United BioSource Corporation, Research Scientist

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following

*statements best describes your view of the public discourse about this effect?
In public discourse, the effect of the pre-market approval policy in preventing harm is...*

...neither understated nor overstated.

*2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?
In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...*

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Insisting on inappropriate or ineffective treatments.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Overreliance on individuals to be fully aware of all relevant information on effectiveness and safety. An impossible task with the amount of information that must be processed.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

Dale H. Gieringer

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Director, California NORML

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Different consumers act differently. Some are systematically over risk-averse, overestimating the risks of adverse side effects. Others are the opposite, underestimating them. Public opinion is always greatly swayed by the latest media stories, which tend not to take a risk-analytic perspective.

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

9a: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

There are many ways that this can happen. I have particular knowledge about the medical use of cannabis in treatment of chronic pain. In this case, a huge proportion of doctors are ignorant of the benefits and fearful of government policy. As a result, they wind up forcing patients off of cannabis and onto prescription narcotics that are far more dangerous.

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

16a: Why not?

If the testing shows that the drug is unsafe and ineffective, obviously it shouldn't be approved. In general, though I prefer a system of informed consumer choice, in which doctors and patients are provided with the appropriate information on drug risks, benefits and uncertainties and allowed to decide for themselves.

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Too many black-and-white yes/no questions.

Paul Greenberg

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Director, Health Care Practice and Managing Principal, Analysis Group, Inc.

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Neutral

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

Paul Grootendorst

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

University of Toronto, Associate Professor

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

ECONOMISTS ON DRUG APPROVAL

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

consumers typically cant correctly use rx drugs without professional supervision

13b: Do you believe that uncertainty per se constitutes a market failure?

No

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

21b: How is it, then, that the public-goods aspect of knowledge provides a rationale for the current system?

FDA approval reduces information search costs to those who might consider using/prescribing the drug. All approved drugs meet some minimum standard of safety and efficacy. That being said, some search is still required to understand which particular therapy is most effective for a given individual.

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

if drug was worse in every aspect compared to already approved therapies, and in particular, was deleterious, then i see no gain in approving it.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

Michael Grossman

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

City University of New York Graduate Center, Distinguished Professor of Economics

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...neither understated nor overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Neutral

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

More possibility for error

13b: Do you believe that uncertainty per se constitutes a market failure?

No

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

21b: How is it, then, that the public-goods aspect of knowledge provides a rationale for the current system?

Spreads costs around

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the

drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

Not sure. Cannot be specific

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

No

László Gulácsi

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Health Economics and Health Technology Assessment Research Centre,
Corvinus University of Budapest, Hungary

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

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...often understated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Neutral

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

13b: Do you believe that uncertainty per se constitutes a market failure?

Yes

14b: How in your view does uncertainty per se constitute a market failure?

Please see textbook of health economics

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Requirements and regulations are very very different

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

Not really systematic but definitely a failure in a number of times - sure

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

No

David R. Henderson

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Associate Professor of Economics, Naval Postgraduate School; Research Fellow, Hoover Institution

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...typically overstated.

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Taking drugs when they don't need to; not taking drugs when they need to. You might think this cancels out, but it doesn't. Some people are systematic in one direction; others in the other direction. Evidence? My personal observation of people around me over the years.

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

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31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Yes. I would have liked to give my reasons for things. Specifically, the question about government's knowledge. We are individuals with different incomes, wealth, inclination, attitudes to risk, etc. The idea that government can make good decisions about what drugs we should have is as absurd as the idea that government can make good decisions about what places we should be allowed to travel to.

Randall Holcombe

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

DeVoe Moore Professor of Economics, Florida State University

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

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10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending

a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

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12a: Do you believe that uncertainty per se constitutes a market failure?

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14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

Yes

18a: How would you describe or explain the government's superior ability to judge safety and efficacy?

Doctors and consumers must get their information somewhere. Doctors typically don't do independent research on medical devices and drugs. Government does, or at least aggregates research results done by others, so it has better information, which it then shares with doctors and consumers. I think independent testing and research labs would do better than government, but that wasn't the question. In fact, government uses results from independent testers, and from the producers themselves, but again, that wasn't the question. I would expect better information from market-based testing organizations, similar in nature to Underwriters Laboratories or Consumer Reports, than from government. But just comparing government to consumers and doctors, government collects more information and is in a better position to judge than consumers or doctors, who (because they are not in a position to do their own research) must get their information from some other source.

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

20a: Why not?

I see no reason why FDA counterparts in other countries would do a better job than the FDA. I favor allowing pharmaceutical firms to market any drugs they want without FDA approval, but I see no reason why the US government should accept approvals by other governments. If the government approval process is flawed, does it make sense to substitute one government's approval process for another's?

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

No.

Charles L. Hooper

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Hoover Institution, visiting fellow

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...typically overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

In cases with any grayness at all, instead of yes/no questions, you might want to consider a five-point scale: strongly agree, agree, neither agree nor disagree, disagree, and strongly disagree. Also, I've found that every question should have a don't know, unclear, decline to state answer because some questions might not be clear to some respondents or some issues might not be resolved. I look forward to the results. Thanks.

John Hornberger

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Cedar Associates LLC, CEO & President

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...neither understated nor overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Neutral

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

5a: Given that you believe there is no sound market-failure rationale for the policy that requires pre-market approval, please explain why you do not oppose the policy.

I am generally impressed by the job FDA does. It is not perfect, but the system is responsive. As it's oversight is congress, politics do come into play, but that is generally unavoidable anyway. For example, it's process for IVDMIA guidance is going too fast.

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

9a: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

It is a principal-agent problem, with MD as the agent for the patient (principal). This system needs the patient to take on more monitoring responsibilities - and they will eventually once the failures of the system become even more manifest.

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

11a: *Why not?*

The patient is not yet able to monitor physician behavior. That time is coming and when so, then I would agree with the idea - in theory.

12a: *Do you believe that uncertainty per se constitutes a market failure?*

No

14a: *Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?*

Yes

15a: *As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?*

No

16a: *Why not?*

If the drug is unsafe, it should not be approved. The FDA is important, expert arbiter of manufacturing quality and safety. Efficacy is an entirely different issue.

17a: *Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?*

No

19a: *As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?*

No

20a: *Why not?*

Incentives are important and US is different.

31: *Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy*

in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

A true market solution - which has yet to be implemented - would involve much more patient input. In testing this idea with colleagues, I was initially surprised by the level of push-back, which primarily justified on the 'asymmetry of information' basis. They simply do not believe 'the average' patient/consumer can acquire the knowledge and judgment needed to be involved in these decisions. The whole set of assumptions applied here need to be examined more carefully.

Don Husereau

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Strategic Advisor, Canadian Agency for Drugs and Technologies in Health

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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...typically overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: *Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?*

Yes

5b1: *Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information*

Imperfect information

5b3: *Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy*

Government has superior ability to assure safety and efficacy

7b: *Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?*

Yes

8b: *In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)*

Information Bias - Leads to inappropriate use of diagnostic testing and harm (Baron, Beattie, Hershey , 1988 and others) Attribution bias - Consumers will incorrectly mental account positive and negative features (Shafir, 1992) Omission bias - Consumers will avoid therapies that benefit Other violations of expected utility related to personal choice under uncertainty - (Tversky and Kahnemann, 1981, 1992; Allais (1953) then Slovic and Tversky (1974) then Keller (1985)

9b: *Do you believe that doctors systematically err when selecting and prescribing therapies?*

Yes

10b: *In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)*

Information Bias - Leads to inappropriate use of diagnostic testing and harm (Baron, Beattie, Hershey , 1988 and others) Attribution bias - Consumers will incorrectly mental account positive and negative features (Shafir, 1992) Omission bias - Consumers will avoid therapies that benefit Other violations of expected utility related to personal choice under uncertainty - (Tversky and Kahnemann, 1981, 1992; Allais (1953) then Slovic and Tversky (1974) then Keller (1985)

11b: *Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending*

a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Because good health care decisions require a more thoughtful approach that is less susceptible to cognitive biases based on incomplete data

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

The question is not about the existence of imperfect information, but who is framing its imperfection. Pre-marketing regulation also requires that uncertainty be framed for the benefit of society, by demanding information that would not otherwise be available.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Because information that indicates an unfavourable choice can be re-framed as favourable for consumers and physicians.

24b: You indicated that a superior ability of government to assure the safety and efficacy of pharmaceuticals justifies the policy requiring pre-market approval. Does that superiority stem from the FDA having special expertise in evaluating safety and efficacy?

No

26b: Would you say that impartiality or commitment to the public good are sources of the government's superior ability to assure safety and efficacy?

Yes

27b: In what ways does the FDA's impartiality or commitment to the public good render it superior to doctors and patients in judging safety and efficacy?

Commitment to a population-based perspective means commitment to population-based data. Consumer-oriented information will mean societal harmful technology can be marketed as good for individuals

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

This scenario assumes that each regulatory system uses the same approach. They do not.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

The questionnaire is not impartial and had a feeling of being leading. It is possible that a person neither believes or disbelieves conceptually and the option was not offered. It seems to be based in an extreme naivety about the power of individuals to make decisions about technology with broad

societal implications. The authors have not seemed to consider the wealth of literature from science and technology studies and scientific philosophy, including Hacking, Shapin, Shaffer, Gallison, Pickering, Graham and others.

John Hutton

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor and Director, York Health Economics Consortium, University of York

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

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...often overstated.

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

I am basing this on personal and professional experience rather than evidence. Some patients tend to underestimate or ignore uncertainties when relying on doctors to make decisions for them. The current system of drug prescription leaves consumers less chance to make errors in choice of treatment. Evidence on adherence to treatment indicates that some patients may not understand the implications of treatment or that doctors may not be correctly interpreting the needs of their patients

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Individual doctors do not have the time or expertise to appraise the evidence on each individual product they might prescribe but many do not follow evidence-based guidelines.

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Would doctors be willing to prescribe in such circumstances? How would they be informed of the characteristics of new treatments? Who would validate the claims of manufacturers? Is the proposal that companies should continue to undertake clinical trials, but no formal assessment of them would

take place? This would not speed market access very much. How would the review process to convert products to OCT differ from the current one?

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Pre -market approval influences the type of information produced by manufacturers. Without it the information needs of informed consumers would be less likely to be met. The way the information is used by the FDA and its peers in other jurisdictions is not always ideal, but that can be changed without removing the requirement to produce the information. In its absence markets for information would no doubt develop. Supplying them would impose costs on manufacturers and health care providers. A priori it is not clear that this would be more efficient.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

I do not regard these as alternatives. However much new knowledge is produced by NIH it has to be developed into products to benefit patients. More co-ordination of the activities of the NIH, the industry and the FDA could be beneficial.

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

This would place information in the public domain. However, such a system would rely on the ability of clinicians (and patients) to determine whether and for whom the treatment would be beneficial. Many aspects of this are generalisable, and probably best done in a co-ordinated rather than a decentralised way. Major health care payers and providers could carry out their own assessments. The end-user would most likely be confronted with a range of information sources with varying degrees of independence. Creating flexibility in FDA decision-making, for example, with more conditional licensing, would be preferable.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Some degree of harmonisation of decisions would be efficient. It could be extended to rejections by other jurisdictions as well as approvals. It has taken a long time for EU countries to accept European level decisions by EMA, and these are still not always accepted by all countries. As long as separate jurisdictions exist there is a danger that regulators will compete to attract applications and the resulting income. If procedures, timescales and fees are standardised the incentive to do this will be reduced, as will the need for separate agencies around the world.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Referring to the previous question, I am not familiar with the detailed record, but I suspect that increased regulation resulted more from concerns about safety. As a general comment, my own view is that decision-making on drugs

and devices would be assisted by more and different information (e.g. on true effectiveness) and by more integration of licensing and reimbursement decisions. From a national health system perspective the problem may be seen as not enough cost-effective products rather than not enough products reaching the market. Changing the way FDA makes decisions would be part of this process e.g. recognising that the trade-off between efficacy and adverse events is one which should be made transparently, and with input from patients. As the focus shifts towards a better-informed reimbursement decision, more FDA decisions would become conditional on further evidence, and its arbitrary ability to halt or delay approval would be reduced. The questionnaire did not allow discussion of these issues which underlay my answers.

Naoki Ikegami

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor & Chair, Dept of Health Policy & Management

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

rational choices are difficult unbiased opinions are difficult

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Based on experience, rather than evidence

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Basic regulations need continue to be maintained

13b: Do you believe that uncertainty per se constitutes a market failure?

No

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

Patients (consumers) cannot necessary digest knowledge

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Why go through the process then?

24b: You indicated that a superior ability of government to assure the safety and efficacy of pharmaceuticals justifies the policy requiring pre-market approval. Does that superiority stem from the FDA having special expertise in evaluating safety and efficacy?

Yes

25b: Why is it that doctors and consumers have inferior judgment in evaluating safety and efficacy?

Difficult to objectively evaluate

26b: Would you say that impartiality or commitment to the public good are sources of the government's superior ability to assure safety and efficacy?

Yes

27b: In what ways does the FDA's impartiality or commitment to the public good render it superior to doctors and patients in judging safety and efficacy?

Impartial, comprehensive

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

If approved in only country, that would be insufficient

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

I cannot cite the evidence. I oppose on conceptual grounds

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Questions are loaded

Michael Iskedjian

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

President, PharmIdeas Research and Consulting Inc.

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...neither understated nor overstated.

*2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?
In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...*

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Neutral

Jonathan Karnon

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor, University of Adelaide

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...*

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: *Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?*

Yes

5b1: *Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information*

Imperfect information

7b: *Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?*

Yes

8b: *In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)*

lifestyle and preventative choices

9b: *Do you believe that doctors systematically err when selecting and prescribing therapies?*

No

11b: *Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?*

No

12b: *Why not?*

imperfect and asymmetric information

13b: *Do you believe that uncertainty per se constitutes a market failure?*

No

17b: *You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?*

No

20b: *Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?*

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

consumers are not best placed to interpret the safety information, and the full burden should not be placed on physicians

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

what happens when there is not a consensus?

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

none

Gordon G. Liu

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor of Economics, Peking University Guanghua School of Management

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often understated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending

a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

without pre-market review and approval, there would be a lack of basis even to classify the products to begin with. If not a government agency like FDA, a societal party must be in place to conduct the review, which is the key issue here, not the agency itself.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

It is important and necessary to pursue a standardized drug safety test and check by a regulatory agency (not necessarily a government agency) before marketing, this is primarily because of the asymmetric information nature of medicine at point of consumption.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

Nikos Maniadakis

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor & Department Head, Health Services Management, National School of Public Health, Athens, Greece

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often understated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

It is very obvious. Perhaps we may lose some of the benefit with the delay but the safety benefit is significant. There are so many technologies which are found to harm at the late stages of pre market approval. What will happen if these enter the market and one or two years down the line are proved to be harmful. There are so many examples of technologies (COX 2 inhibitors) that are proven to have issues even with the existing structures in place. The risk would be high and the cost would be unacceptable.

13b: Do you believe that uncertainty per se constitutes a market failure?

Yes

14b: How in your view does uncertainty per se constitute a market failure?

It is generally a failure because consumers in this era are not like other consumers. I do admit though that nowadays, because of information, knowledge, institutions, providers and organisations in place, is not so much an issue as it was in the past.

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Risk taking behaviours of some providers which are trying to maximise financial and related objectives in the short run.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

It can be part of the picture but can not be the solution. Who says that public institutions and their staff are doing the job better than the market?

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Because is not enough by any means. There so little we know about their effects in that stage. It is so early in the process and the knowledge is limited, on average.

24b: You indicated that a superior ability of government to assure the safety and efficacy of pharmaceuticals justifies the policy requiring pre-market approval. Does that superiority stem from the FDA having special expertise in evaluating safety and efficacy?

Yes

25b: Why is it that doctors and consumers have inferior judgment in evaluating safety and efficacy?

Many reasons. Lack of data, expertise, time, sources, ability and many others. Can they replace an institutions such as FDA. Of course it needs to be pointed that their evaluations are important too, but later on on top of preceding assessments.

26b: Would you say that impartiality or commitment to the public good are sources of the government's superior ability to assure safety and efficacy?

Yes

27b: In what ways does the FDA's impartiality or commitment to the public good render it superior to doctors and patients in judging safety and efficacy?

I am not arguing that this is always the case and that there are no exemptions to the impartiality and commitment. However, this is primarily the objective and in general is being achieved, not so much because of the impartiality but because of the size and expertise and resources.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Liek so many things in life, medical science is not black or white and objective. It is down to the individual country and its experts, on the basis of many factors and considerations to judge whether a new technology is safe and efficacious for the specific population, relative to what is already available and known.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: *Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?*

No

34: *Do you have any general comments about this questionnaire or further thoughts about the matters treated?*

NO

Karl A. Matuszewski

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Vice President, Editor-in-Chief, Gold Standard/Elsevier

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

US health care system subject to irrational decision-making due to conflicting forces dominated by income maximization

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

tend to self diagnosis, susceptible to marketing influences

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

General inability to keep current with medical literature and insidious effects of technology marketing and educational support

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Anecdotal experience would replace scientific knowledge and such a system would be highly susceptible to industry influences that might not be in the best interests of patient care.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Health care in US not universal, but rather subsidized in an efficient manner. Access and use of medical technologies varies widely across the country, along with expected outcomes. Cottage-industry structure of health care delivery and financing leads to market failures for large segments of the population

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

The safety of the general public requires the ability to determine that certain risks are too serious to allow

ECONOMISTS ON DRUG APPROVAL

24b: You indicated that a superior ability of government to assure the safety and efficacy of pharmaceuticals justifies the policy requiring pre-market approval. Does that superiority stem from the FDA having special expertise in evaluating safety and efficacy?

Yes

25b: Why is it that doctors and consumers have inferior judgment in evaluating safety and efficacy?

Lack of focus on pure unbiased evaluation, general lack of time and expertise to conduct such evaluations, and large marketing pressure from technology industry, clinical peers, and patient advocacy groups.

26b: Would you say that impartiality or commitment to the public good are sources of the government's superior ability to assure safety and efficacy?

Yes

27b: In what ways does the FDA's impartiality or commitment to the public good render it superior to doctors and patients in judging safety and efficacy?

No conflicts economically and able to consider societal perspective.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

US FDA process is still considered by most of the world as gold standard, which I believe it is.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

large number of products eliminated after DESI review

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

No

C. Daniel Mullins

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor, University of Maryland School of Pharmacy

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...neither understated nor overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Patients infrequently use evidence-based decision making strategies Many patients are not able to comprehend and adhere to treatment recommendations (e.g. non-compliance, over/under dosing) Most patients excessively discount future risks

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Although many physicians practice evidence-based medicine, marketing by branded pharmaceutical manufacturers introduces a systematic bias towards prescribing of branded medicines, even when there is insufficient evidence of superiority to non-advertised therapeutic equivalents.

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Physicians do not have the time or resources to evaluate the enormity of evidence that allows a systematic review of the benefits and risks of drugs.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

The asymmetry of information between pharmaceutical manufacturers on the one hand and prescribers, patients, and other decision makers on the other hand reflects a market failure. This is counter-balanced in the current

environment by FDA policy that requires manufacturers to promote their products according to the FDA-approved label.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

No

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

There are many terminally ill patients who would be harmed by unsafe products that may be inaccurately viewed as providing sufficient potential value in terms of the possibility of cure.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

First, the other regulators could make incorrect decisions. Second, there could actually be scientific reasons why the benefit/risk trade-off of a drug would differ across patient populations.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: *Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?*

Yes

33: *What evidence can you cite for systematic failure in assuring efficacy prior to 1962?*

The lack of evidence of efficacy in my mind equates to evidence of systematic failure. That is the concept of the null hypothesis: the product is assumed to have a value of zero until proven efficacious at $p < 0.05$.

34: *Do you have any general comments about this questionnaire or further thoughts about the matters treated?*

No

Sam Peltzman

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

prof emeritus u of chicago

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

Charles E. Phelps

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

University of Rochester

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Neutral

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Doctors also have limited information and highly costly time to acquire it. Widespread regional variations in medical practice styles tell us that doctors do not uniformly agree on what the best practice patterns are. I could easily see widespread damage from the model you suggest. If liability law could guarantee recovery from damage, I might change my mind, but the possibility of bankruptcy limits the effectiveness of such a process in reality. Drugs have such widespread use that liability rules could have little meaning, especially for smaller drug companies.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

Again, I fear that if we abandoned pre-market approval, liability rules could often fail as a deterrent to drug makers. In addition, the public good aspect of knowledge entails both the production of knowledge and acquisition and use by consumers (or their agents, doctors). The latter is costly, no matter

how much the NIH supports knowledge generation. Stigler's economics of information work led the way to thinking about this.

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Same story. The idea presumes that if a drug tested and came out unsafe that people would not use it and doctors would not prescribe it. This presumes that the acquisition and use of the information is (almost) costless, which is demonstrably wrong (again, see the medical practice variations literature).

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

I could favor this idea, with precondition about the list of countries with whom we had reciprocity. If the idea (which your phrasing suggests) is that if ANY country outside the US approves it (you said Europe, does that include Monaco?) then you would have a game theoretic problem where a country could become the approver of choice for drug companies. But if the idea were a list of pre-arranged reciprocity arrangements, I would support it. That would keep control of the approved list in our hands, which is where it should be. We should not have foreign nations determining our domestic (regulatory) policy without our approval.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

Finally I got to a Please explain on my no answer to the efficacy question! It's not a systematic failure that led me to say no on the 1962 efficacy requirements, it's (once again) the issue of the costs of information to patients and doctors, combined with the potential failure of tort law. It combines with the question about false claims by drug companies and the costs of having the market police those claims. There is a real public good aspect to finding out if a drug systematically fails to work, and false claims (coupled with an ineffective tort system for redress) make me worry about eliminating the efficacy claims too.

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

You seem to have overlooked several alternatives to yes or no on the FDA and its processes. David Weimer, now at the University of Wisconsin, once proposed what I thought was an excellent way to get rid of most of the FDA apparatus, and it solves my concerns about liability law failure. He suggested that to market a drug in the US, drug makers would have to put money into a pool to compensate harmed individuals and then let post-marketing surveillance find out about safety and efficacy. Your questionnaire did not give latitude to think about such alternatives.

G rard de Pouvourville

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor at ESSEC Business School, Chair of Health Economics and Management

1: *The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).*

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: *Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?*

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: *Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?*

Support strongly

4: *Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?*

Yes

5b1: *Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information*

Imperfect information

5b4: *Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other*

Other

6b: *Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.*

Not only imperfect information, but also complexity of information and need to protect the consumer.

7b: *Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?*

No

9b: *Do you believe that doctors systematically err when selecting and prescribing therapies?*

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

There is not enough details in this proposal to adopt it as such: how thorough would the classification scheme be? Moreover, this would be getting back to old ages of panaceas sold without control. Medicines have grown too complex not to be controlled for their innocuity and efficacy.

13b: Do you believe that uncertainty per se constitutes a market failure?

Yes

14b: How in your view does uncertainty per se constitute a market failure?

The question is ambiguous: most importantly it is not uncertainty per se alone but the asymmetric capacity of actors to deal with it, leading to opportunistic strategies which could kill people in the domain of medicines.

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Again the question is as much imperfection as it is asymmetry of knowledge: even if Doctors are more and more educated they cannot cope with the growing complexity of knowledge required to develop a new drug.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

This question is again ambiguous, and does not allow for a yes or no answer. What does regardless of what those testing results turned out to be? mean? So I answer no because I do not understand either what an automatic approval would be unless it does not check for a correct balance of benefits and risks!

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

Sorry there is no place for an I don't know answer! This is what I would have said.

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Yes I do have some comments on the forced choices and on the labelling of propositions that do not allow for a correctly structured argumentation. FDA may be criticized and its processes may be too cumbersome, but there is no way one should go back to a situation when drug companies were marketing drugs the safety and efficacy of which were not supported by good evidence. Perhaps there is a growing aversion to risk in our western societies which may impede the development of innovations, but this has to be carefully assessed.

José Luís Pinto Prades

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor in Economics University Pablo de Olavide Sevilla Spain

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

ECONOMISTS ON DRUG APPROVAL

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

They change both efficient and inefficient consumption in reaction to financial incentives

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

I do not think that doctors can gather as much information as a more centralized system in order to take good decisions. I think doctors need help in processing and understanding clinical evidence.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

Paul H. Rubin

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Samuel Candler Dobbs Professor of Economics, Emory University

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...typically overstated.

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In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

People overestimate risks of drugs and underestimate benefits.

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending

a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

16a: Why not?

If we force pharmaceutical companies to go through the process, we should use the information generated.

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

F.M. Scherer

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Aetna Professor Emeritus, John F. Kennedy School of Government,
Harvard University.

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

It has also been historical fact that, despite the threat of significant tort liability for harmful drugs and devices, manufacturers have been less than diligent in ensuring that pre-market testing is adequate and that public health hazards are avoided.

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Most consumers lack the information needed to evaluate the drugs they take; indeed, many don't even read the FDA-required label indications. It has also been my experience, marked to be sure by some pleasant exceptions, that drug prescribers are so harried that they do not consider seriously all relevant safety and efficacy effects. I personally have had ulcers from ill-prescribed drugs and was once prescribed drug therapy for what turned out to be a serious cancer. I once attended a University of Chicago seminar at which a physician described the exquisite care he applied in choosing drugs for his patients. I sat there thinking, My God! Where do I find such a physician?

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Your earlier question asked whether physicians systematically err; I replied no. But they do err randomly, and frequently, in large measure because they are simply so busy that they don't have time to consider indications and

contra-indications. Also, there are informational economies of scale in having an agency like the FDA consider in detail, however imperfectly, all the evidence available on indications and contra-indications. Most physicians simply don't have time to do this.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

To repeat, there are informational economies of scale.

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

The question was, better addressed, implying that NIH would substitute for what FDA does, as Marsha Angell has suggested. NIH makes wonderful contributions to the generation of health care knowledge. But to add the kinds of responsibilities FDA now carries would not exploit its comparative advantage and indeed might even for bureaucratic reasons be less than advantageous than having an agency whose principal mission is ensuring safety and efficacy.

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

The historical record is clear: FDA has made serious errors, and physicians have in large numbers prescribed the approved drugs. So the system is imperfect. But without the informational function the FDA performs, there would be even more prescribing errors.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Well, we in the United States are big guys (to be sure, like the Europeans), better able to exploit the informational economies of scale. And given the inevitability of agency error, it's good to have several responsible regulatory agencies around the world, so that errors by one may be corrected by others' correct findings. The same logic applies for decentralizing regulatory functions to the individual states, but in this case, the informational economies of scale call for more centralization. Creation of a central European drug approval agency reflected that tradeoff.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

Only casual historical reading plus having lived through part of the pre-1962 period: prescription of ineffective medicines was rampant.

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

Well, it has an implicit point of view, but viva le difference!

David A. Sclar

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Boeing Distinguished Profesor of Health Policy and Administration
Washington State University

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support, not strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Over or under utilization of services; moral hazard

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Over or under utilization; moral hazard; concern re: malpractice

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Insufficient [uniformity of] education among MDs. Patient forum shopping.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Lack of tort reform

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

No

19b: Why not?

Inherent bias at the NIH vs. competitive market forces (private firms)

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Not absent tort reform; MD education; patient education re: risk/benefits (known and unknown)

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

Yes

33: What evidence can you cite for systematic failure in assuring efficacy prior to 1962?

The concern is not pre-1962. Rather with the proliferation of new agents (natural products; synthetic; biotech) efficacy data is essential to rational prescribing

Robert M. Sigmund

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Walter J McNerney Fellow, HRET, American Hospital Association

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In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...neither understated nor overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

5b2: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Public-goods aspects of knowledge

Public-goods aspects of knowledge

5b3: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Government has superior ability to assure safety and efficacy

Government has superior ability to assure safety and efficacy

5b4: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Other

Other

6b: Please describe the 'other' source of market failure that justifies the policy requiring pre-market approval.

Conflicts of special interests with the public interest

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Assuming that professionals always know what's best

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

10b: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

Doctors are not able to keep up with the latest new knowledge

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Too dependent on physicians who do not know enough and who may have conflicts of interest

13b: Do you believe that uncertainty per se constitutes a market failure?

No

18b: You indicated that public-goods aspects of knowledge are a source of the market failure that justifies the policy requiring pre-market approval. Do you think that this source of market failure would be better addressed with a policy that subsidizes the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

Because the testing results might be too negative

24b: You indicated that a superior ability of government to assure the safety and efficacy of pharmaceuticals justifies the policy requiring pre-market approval. Does that superiority stem from the FDA having special expertise in evaluating safety and efficacy?

Yes

25b: Why is it that doctors and consumers have inferior judgment in evaluating safety and efficacy?

Neither the doctors nor the patients have the expertise and the time to get all of the information required for better judgment

26b: Would you say that impartiality or commitment to the public good are sources of the government's superior ability to assure safety and efficacy?

Yes

27b: In what ways does the FDA's impartiality or commitment to the public good render it superior to doctors and patients in judging safety and efficacy?

These factors provide the FDA with more time and resources and also minimize the possibility of personal conflicts of interest

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

30b: Why not?

Because we do not know enough about how the FDA counterparts for automatic approval

31: *Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?*

No

32: *Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?*

No

34: *Do you have any general comments about this questionnaire or further thoughts about the matters treated?*

The market would probably function better in the public interest if advertising of prescription drugs were not permitted, as was the practice when Rufus Rorem wrote his great book on the pharmaceutical industry for the Committee on the Costs of Medical Care in the early 1930's. I have a rare copy.

Shirley Svorny

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor of Economics, California State University, Northridge

1: *The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).*

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...typically overstated.

2: *Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?*

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

7a: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

If you use economic efficiency as a benchmark, you have to say that consumers consistently err in consuming services for which the marginal benefit is less than the marginal cost. Most consumers don't see prices when they buy health care. In addition, favorable tax treatment for employer-purchased health insurance leads to smaller deductibles and coinsurance rates. This encourages consumers to buy services for which the marginal benefit is less than the actual marginal cost (consumers do not bear this cost).

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

Yes

9a: In what ways does doctor erring manifest itself? (If possible, please cite relevant evidence.)

With third party insurance, doctors are likely to recommend procedures for which the marginal benefit is less than the marginal cost.

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

16a: Why not?

The FDA processes may be excessive. Why not allow innovation in processes as you would expect if assurance came from brand name or private certification organizations. I want to say something re # 14a: I really wanted to say it depends as some products may go to the market with less investigation and some with more, it would depend on what information consumers want or what information companies (legally liable) or certification organization might want. It could generate more or less knowledge than in the absence of the policy.

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

Yes

18a: How would you describe or explain the government's superior ability to judge safety and efficacy?

Compared to individual physicians and consumers, the government can do better. BUT if there is brand name or private certification that individual physicians and consumers can rely upon, then I believe physicians and consumers would have access to better information.

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

No

20a: Why not?

I'd rather have market forces (brand name and private certification) inform consumers. I worry that government agencies, including the FDA, may choose criteria that do not maximize consumer welfare.

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for "off-label" use—that is, for use where there has been no FDA evaluation of the drug's efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug's "on-label" use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

My co-author (cited by Klein), Kathleen Johnson, is not an economist (she is on your list). She is a pharmacist with a Ph.D. in public health (or something like that). If the FDA process were eliminated, major brand names, like Bayer and Tylenol, would increase in market value. They have the reputation to bring forth new drugs. The same holds true for major drug companies that already have good reputations with physicians and/or consumers.

Robert Tollison

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

dept of economics, clemson university, J. Wilson Newman Professor of Economics

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

No

6a: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

8a: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

10a: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

11a: Why not?

My view is that testing protocols need to be deregulated and designed to work faster.

12a: Do you believe that uncertainty per se constitutes a market failure?

No

14a: Knowledge is often said to have public-goods aspects. Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

15a: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

16a: Why not?

Screen out losers if identified ex ante.

17a: Relative to doctors and consumers, do you believe the government has superior ability to judge the safety and efficacy of pharmaceuticals (assuming, that is, that the government does not have exclusive access to certain research information)?

No

19a: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

34: Do you have any general comments about this questionnaire or further thoughts about the matters treated?

I support a halfway house between what we have now and a faster way to market for new drugs, in which approved foreign drugs would be allowed and the testing protocols we have today would be liberalized. For more see Tollison, Institutional Alternatives for the Regulation of Drugs and Medical Devices, in *Advancing Medical Technology, Progress and Freedom* Foundation, 2/7/1996.

Michael R. Ward

B: As stated in the contact email, your responses will be reported as yours, without anonymity. Please confirm this understanding:

Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

University of Texas at Arlington

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...often overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...typically understated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Oppose strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

No

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

Yes

13b: Do you believe that uncertainty per se constitutes a market failure?

No

15b: If neither consumers nor doctors err systematically in these matters and uncertainty per se does not constitute a market failure, then in what sense do you believe that imperfect information is a source of the market failure that justifies the policy requiring pre-market approval?

I do not. The survey questions are misleading. In the standard jargon, asymmetric information constitutes a market failure and therefore I agree that there exists a market-failure rationale for the policy. There are also competing arguments against the policy. Agreeing that a market failure exists does not imply that I agree that they justify the policy.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

Yes

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

No

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

Yes

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

Yes

Albert I. Wertheimer

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Yes, I understand that my responses will be made public (accurately, completely, and neutrally) and without anonymity.

C: Please give your current institutional affiliation and title:

Professor, Temple University

1: The policy that requires pre-market approval of new pharmaceuticals and medical devices may prevent harm, and it also may suppress would-have-been benefits (by suppressing drug development, causing delays, or increasing costs).

Consider for the moment only the first of these two policy effects, preventing harm. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in preventing harm is...

...neither understated nor overstated.

2: Consider now only the second policy effect, suppression of would-have-been benefits. Which of the following statements best describes your view of the public discourse about this effect?

In public discourse, the effect of the pre-market approval policy in suppressing would-have-been benefits is...

...often overstated.

3: Which best describes your attitude toward the U.S. government policy that requires pre-market approval of new pharmaceuticals and devices?

Support strongly

4: Do you think there is a sound market-failure rationale for the policy that requires pre-market approval?

Yes

5b1: Which of the following describes the nature or source of the market failure that justifies the policy requiring pre-market approval? Please be sure to check any and all that you believe to apply. -Imperfect information

Imperfect information

7b: Do you believe that consumers or patients systematically err when coping with uncertainties related to health and treatment?

Yes

8b: In what ways does consumer or patient erring manifest itself? (If possible, please cite relevant evidence.)

Purchasing OTC advertised on TV, and asking MD for Rx drugs seen in advertisements.

9b: Do you believe that doctors systematically err when selecting and prescribing therapies?

No

11b: Say the policy that requires pre-market approval was eliminated and, in its place, a policy was implemented allowing new pharmaceuticals/ devices and initially classifying them as requiring a doctor's prescription (pending a review process to consider dropping the prescription requirement). Do you think such a liberalized system would be superior to the current system?

No

12b: Why not?

Too few prescribers see enough patients to be able to make informed, objective decisions; and no control group.

13b: Do you believe that uncertainty per se constitutes a market failure?

No

16b: In addition to systematic error on the part of consumers/ doctors and/ or uncertainty per se, is there any other sense in which you believe imperfect information to be a source of the market failure that justifies the policy requiring pre-market approval? If so, please explain.

Adverse event reporting is not often done due to potential risk of litigation.

17b: You indicated that public-goods aspects of knowledge do not justify the policy requiring pre-market approval. Is that because you think such aspects are better addressed by subsidizing the generation of knowledge, e.g., via the National Institutes for Health?

No

20b: Imagine a new pharmaceutical being developed within the current regulatory system and brought all the way through to FDA approval. Do you think the policy that requires pre-market approval induces the generation of more knowledge about the new pharmaceutical than there would have been in the absence of the policy?

Yes

22b: As compared to the current system, would you favor making approval of a new drug automatic upon completion of the safety and efficacy testing processes specified by the FDA, regardless of what those testing results turned out to be?

No

23b: Why not?

I prefer HTA and/or Comparative Effectiveness Research to identify the optimal therapy.

29b: As compared to the current system, would you favor a reform so that pharmaceuticals approved by the FDA counterparts in Europe, Japan, Canada, or Australia were automatically approved for the United States?

Yes

31: Prior to 1962, the FDA did not consider efficacy when making drug approval decisions. Doctors today are at liberty to prescribe drugs for “off-label” use—that is, for use where there has been no FDA evaluation of the drug’s efficacy. However, as part of the current pre-market approval process, the FDA requires proof of efficacy in the drug’s “on-label” use(s). Would you favor dropping efficacy requirements from the pre-market approval process?

No

32: Efficacy requirements were introduced in 1962. Do you believe that the pre-1962 record shows systematic failure in assuring efficacy?

No

Handy Links:

- [The January 2010 call to 305 economists](#)
- The questionnaire is in two forms: [HTML](#) (interactive), [PDF](#) (noninteractive)
- [The May 2010 report on the 44 responses compiled in the present document](#)
- [The present transcripts in spreadsheet form](#)



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