

Supplementary Online Content

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eAppendix. Full Text and Layout of Survey Questions

This supplementary material has been provided by the authors to give readers additional information about their work.

eAppendix. Full text and layout of survey questions

1. FDA approval typically means that a drug...

		True	False
a.	is as effective as other drugs approved to treat the same condition.	<input type="radio"/>	<input type="radio"/>
b.	has benefits that outweigh its harms.	<input type="radio"/>	<input type="radio"/>

2. In order for a drug to get FDA approval it has to have:

- A statistically significant result.
- A clinically important result.
- Both of the above.
- None of the above.

FDA has recently started designating some new prescription drugs it reviews and approves as “breakthrough drugs”.

3. Before taking this survey, how familiar were you with the “breakthrough drug” designation?

- Very familiar
- Familiar
- A little familiar
- Not familiar at all

4. In general, I am certain that an FDA-approved “breakthrough drug” represents a major advance over currently-approved treatments for its indication. [Pick one option below]

- Very certain
- Fairly certain
- Fairly uncertain
- Very uncertain

5. What is the minimum level of evidence that FDA requires manufacturers to gather in order for the FDA to label a drug as a “breakthrough”? [Pick one option below]

- Strong evidence (e.g., randomized trials evaluating clinical outcomes)
- Preliminary (e.g., uncontrolled studies or studies testing surrogate outcomes)
- Very preliminary (e.g., *in vitro* lab or animal studies)

6. When FDA calls a drug a “breakthrough,” does that mean that there is high quality evidence that the drug is...

a	more effective than currently approved treatments?	<input type="radio"/>	<input type="radio"/>
b	safer than currently approved treatments?	<input type="radio"/>	<input type="radio"/>

7. Imagine your patient has a serious medical condition for which there has been no effective treatment. FDA recently approved 2 new drugs to treat this condition. Both drugs are oral tablets to be taken once a day, have similar side effect profiles, and are equally covered by the patient's insurance. Which would you choose first?

- Axabex, a FDA-designated "breakthrough" drug
- Zykanta, a drug with early promising study results but which has not been shown to improve survival or disease-related symptoms.

8. RANDOMIZED HYPOTHETICAL

Group 1 (Facts Alone version): In June 2014, the FDA approved Procampa for lung cancer patients resistant to other treatments. Drug approval was based on a clinical trial of 163 patients with a type of lung cancer that usually does not respond to treatment. All participants were treated with Procampa. Results showed that about half of the participants had their tumors shrink, and this effect lasted an average of about seven months. Common side effects of Procampa include gastrointestinal symptoms such as diarrhea, nausea, vomiting and abdominal pain. Laboratory abnormalities such as increased liver enzymes, pancreatic enzymes and increased glucose levels were also observed.

Group 2 (Breakthrough version): In June 2014, the FDA approved Procampa for lung cancer patients resistant to other treatments and designated it as a "breakthrough drug". Drug approval was based on a clinical trial of 163 patients with a type of lung cancer that usually does not respond to treatment. All participants were treated with Procampa. Results showed that about half of the participants had their tumors shrink, and this effect lasted an average of about seven months. Common side effects of Procampa include gastrointestinal symptoms such as diarrhea, nausea, vomiting and abdominal pain. Laboratory abnormalities such as increased liver enzymes, pancreatic enzymes and increased glucose levels were also observed.

Group 3 (Breakthrough/Expedited version): In June 2014, the FDA approved Procampa for lung cancer patients resistant to other treatments and designated it as a "breakthrough drug". The approval was expedited and occurred four months ahead of the FDA's review completion goal date. Drug approval was based on a clinical trial of 163 patients with a type of lung cancer that usually does not respond to treatment. All participants were treated with Procampa. Results showed that about half of the participants had their tumors shrink, and this effect lasted an average of about seven months. Common side effects of Procampa include gastrointestinal symptoms such as diarrhea, nausea, vomiting and abdominal pain. Laboratory abnormalities such as increased liver enzymes, pancreatic enzymes and increased glucose levels were also observed.

Group 4 (Breakthrough/Warning version): In June 2014, the FDA approved Procampa for lung cancer patients resistant to other treatments and designated it as a "breakthrough drug". The drug's label includes a warning than an improvement in survival or disease-related symptoms

has not been established. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trials. Drug approval was based on a clinical trial of 163 patients with a type of lung cancer that usually does not respond to treatment. All participants were treated with Procampa. Results showed that about half of the participants had their tumors shrink, and this effect lasted an average of about seven months. Common side effects of Procampa include gastrointestinal symptoms such as diarrhea, nausea, vomiting and abdominal pain. Laboratory abnormalities such as increased liver enzymes, pancreatic enzymes and increased glucose levels were also observed.

Please respond to the following statements. Assume that all of your patients with lung cancer have insurance plans that fully cover the cost of Procampa.

	Definitely yes	Probably yes	Probably no	Definitely no
8a. If I had patients with late-stage lung cancer, I would suggest they try Procampa.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
8b. I believe Procampa improves survival for patients with late-stage lung cancer.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
8c. I believe that there is strong evidence of Procampa's benefit to patients with late-stage lung cancer.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>