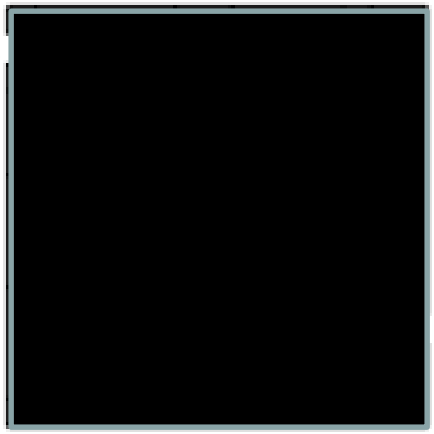
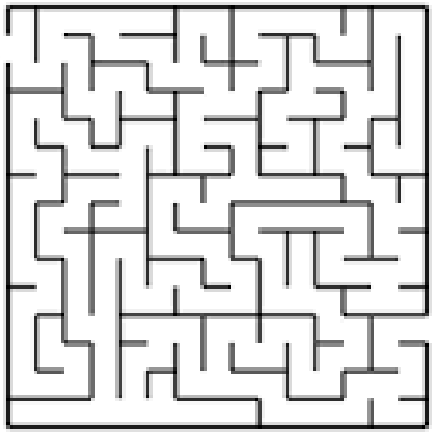
Good idea



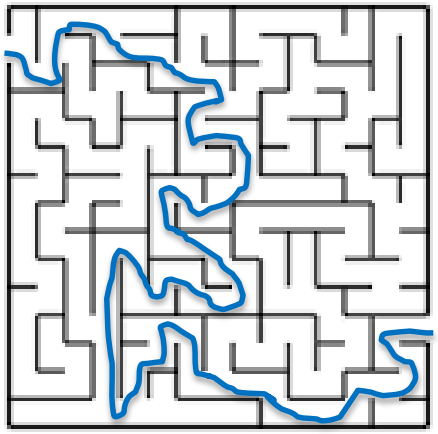
Approved drug/biologic

Good idea



Approved drug/biologic

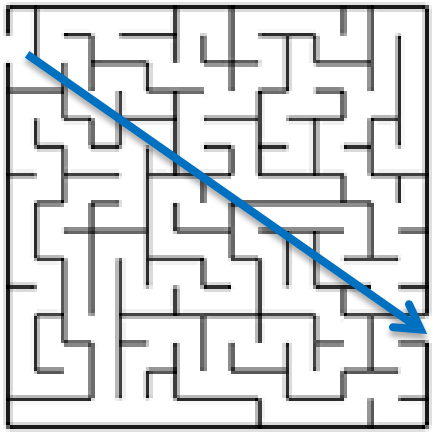
Good idea



Approved drug/biologic

Are There Alternative Regulatory Pathways?

Good idea



Approved drug/biologic

Accelerated Approval Pathway

- When “routine” clinical studies are impractical or unethical (e.g. mortality with slow growing tumor or with rare clinical events) ✓
- Requires the same statutory standards for safety and efficacy as a routine application
- A surrogate endpoint – or an intermediate clinical endpoint – likely to predict clinical benefit ✓
  - There is precedent for using laboratory data as surrogate (Factor Xa reversal agent)
- Postmarketing confirmatory trials are required to verify and describe the anticipated effect on surrogate or intermediate clinical endpoint or other clinical benefit

Accelerated Approval Pathway

- Serious Condition ✓
- Meaningful advantage over available therapy ✓
- Effect on endpoint that is reasonably likely to predict clinical benefit
  - Clinical data should be provided to support a conclusion that a relationship of an effect on the surrogate endpoint or intermediate clinical endpoint relates to an effect on the clinical outcome and is reasonably likely.
- Confirmatory trial(s) should directly measure clinical benefit and be underway at the time of submission of original application

Resources

- FDA Guidances
  - Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics, May 2014
  - Guidance for Industry: Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products, May 1998
- Questions and Answers about the Expedited programs can be found at: http://www.fda.gov/regulatoryinformation/legislation/federalfooddrugandcosmeticactfdcact/significantamendmentstothefdcact/fdasia/ucm341027.htm