THE PEW CHARITABLE TRUSTS

"Characteristics of Pivotal Trials and FDA Review of Innovative Devices"

PLOS ONE

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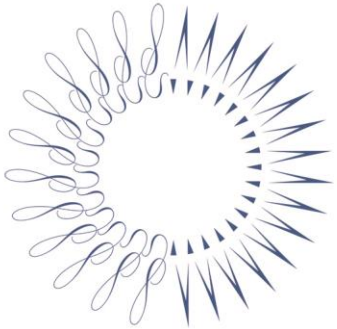
The Pew Charitable Trusts

Pew is an independent, non-profit research and public policy organization.

Pew seeks to enhance medical device safety and foster device innovation that benefits patients.

- Unique device identifier (UDI)
- Medical device registries
- Accelerating device innovation



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Questions and comments appear here

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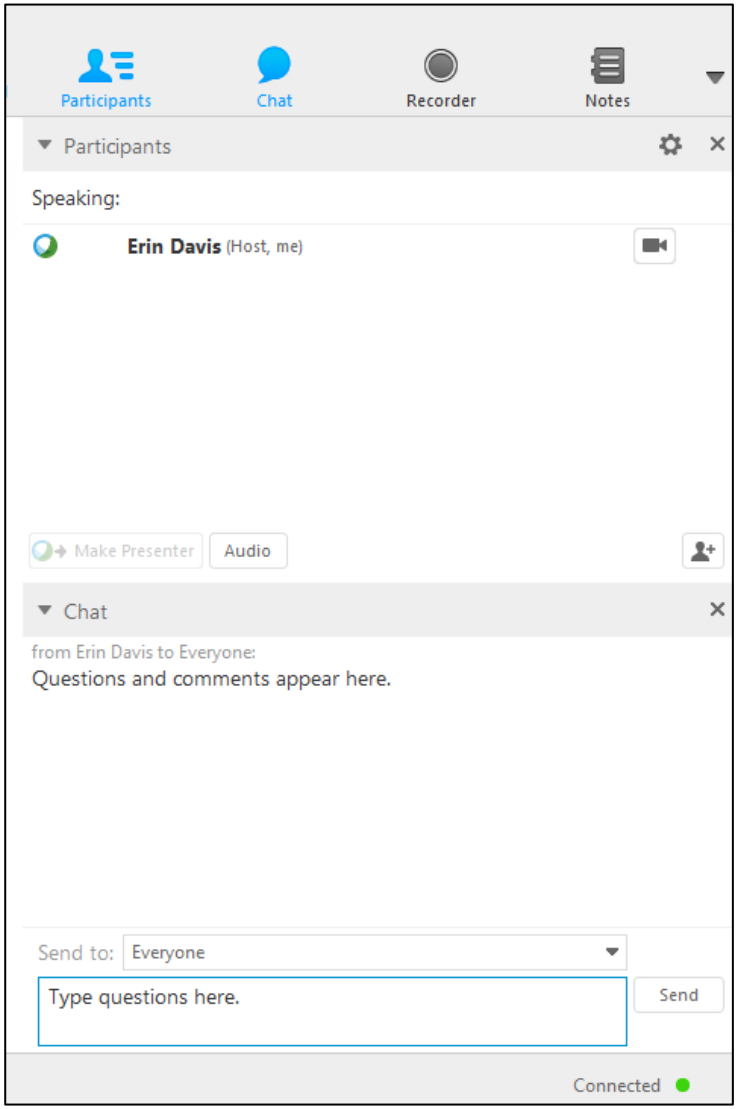


Webinar Agenda

- Research overview
  - Ben Moscovitch, Officer, Medical Devices, The Pew Charitable Trusts

- Moderated questions with a panel of experts

Background: Efforts to Shorten Clinical Development

- Congress examining policies to spur innovation
  - 21st Century Cures
  - Recent Senate HELP Committee Report

- FDA Expedited Access PMA (EAP) initiative
  - Applicable to innovative devices for serious, unmet needs
  - Would shift some data collection to the postmarket setting
    - Shorter follow-up in the premarket setting
    - Reliance on surrogate endpoint
    - Smaller trial
  - Requires postmarket controls

Research Purpose

- Evaluate how long it takes innovative medical devices to reach patients
  - Length of pivotal clinical trial
  - Length of FDA review

- Examine other characteristics of the pivotal clinical trials
  - Enrollment
  - Length of primary outcome measure

Scope and Methodology

Scope

- Restricted to approved priority review applications
  - FDA grants priority review designations for products that address serious, unmet medical needs
  - Moves these applications to the front of the review queue
  - Does not change the approval standard

- Limited to premarket approval (PMA) devices

- Date range: Jan. 2006 through Aug. 2013

Scope and Methodology, cont.

Data sources

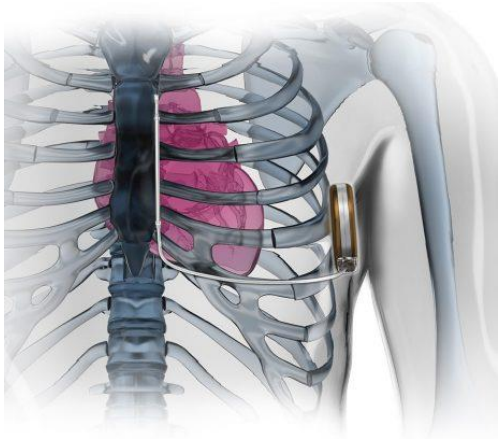
- Sources for approved devices:
  - Summary of Safety and Effectiveness Data (SSED)
  - ClinicalTrials.gov
  - Advisory committee documents

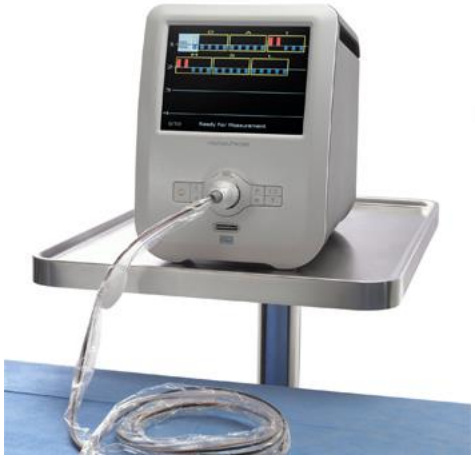
- FDA provided some aggregate information on unapproved applications

Data Sought

- **For each product:**
  - Length of pivotal trial
  - Length of primary outcome measure
  - Time between submission to FDA and approval*
  - Number of enrollees in the pivotal trial
  - Availability in US first

What Type of Products





Key Findings

- **27 approved priority review devices identified**
  - Two devices lacked complete information and were not included in all calculations

- Pivotal trial
  - Median duration: **3 years** (3 months to 7 years)
    - Mean duration: 3 years, 5 months
  - Median primary outcome time: **1 year** (6 days to 3 years)
    - Mean time: 1 year
  - Median enrollment: **297 enrollees** (42 to 2526)
    - Mean enrollment: 663 enrollees

Key Findings, cont.

- Median FDA review time: **1 year, 3 months***
  - Mean: 1 year, 5 months

- **75 percent** of new devices were available abroad first

- **11 devices (29 percent)** were not approved

Limitations

- **Not all data was uniformly depicted or was missing from SSED**
  - Used multiple data sets to fill in gaps
- **Data only available on approved devices**
  - FDA supplemented the findings with aggregate information
- **Unclear the details on why products were unapproved**
- **FDA review time includes when FDA awaits more information**

Conclusions

- **Clinical trials are much longer than FDA review**
  - This is the area to target reforms without sacrificing standards
- **Primary outcome measures** are much shorter than the trials
- **Majority of devices available abroad first**
  - Experts must identify ways to expedite access in the United States without endangering patients

Conclusions, cont.

- **Approximately one-third of devices not ultimately approved**
  - If FDA shifts data collection, it is possible that some of these devices would have been approved
  - Data must be collected postmarket
  - Strict controls are essential for FDA to withdraw approval if data aren't collected or product is not safe/effective

Policy Implications

- Expedited Access PMA
- Other potential policies or legislation

<u>These policies should:</u>

1) Target areas where significant progress can be made
2) Uphold standards to avoid approvals of unsafe/ineffective devices
3) Restrict these efforts to those devices that will significantly improve the standard of care for patients with serious conditions
4) Ensure the postmarket data collection infrastructure is in place
   - Registries
   - UDI capture in patients health records and claims

Q&As with Panelists

- Owen Faris, Acting Clinical Trials Director, Center for Devices and Radiological Health, FDA
- Bray Patrick-Lake, CEO, PFO Research Foundation
- Larry Wood, Corporate Vice President, Transcatheter Heart Valves, Edwards Lifesciences
- Ben Moscovitch, Officer, Medical Devices, The Pew Charitable Trusts
- Moderator: Josh Rising, Director, Healthcare Programs, The Pew Charitable Trusts

Participate!

- Type in your question or comments

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Thank you

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