

# Enhancing Drug and Device Safety and Effectiveness

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before the bipartisan HELP Innovation Working Group  
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# CLINICAL TRIAL DATA TRANSPARENCY

# Benefits of FDA Data Transparency

- Find new information on safety and effectiveness
- Bring to light problems not detected during rapid FDA reviews
- Combine data sets to learn how rare sub-groups respond, advancing personalized medicine
- Avoid unnecessary clinical trials, saving costs and protecting human subjects from potentially harmful experiments

# Data Transparency Momentum

- The Institute of Medicine recommended that the FDA share full CSRs (redacted for commercial or personal info) for all drugs, after approval or final abandonment.
- The European Medicines Agency (EMA) already shares clinical trial data on drugs approved in Europe.
- A White House memorandum urges agencies to maximize the public availability of information arising from federally funded research.
- The National Institutes of Health requests data sharing from recipients of federal grants.
- Recent legislation to promote greater data sharing includes: HR 2031 (2013) and HR 617 (2015).

# Our Proposal

We support enhancing existing data sharing mechanisms through legislation to mandate publication of all CSRs and related electronic data sets (with personal info and trade secrets redacted) after drug is finally approved or rejected by the FDA.

# SPEED VERSUS SAFETY

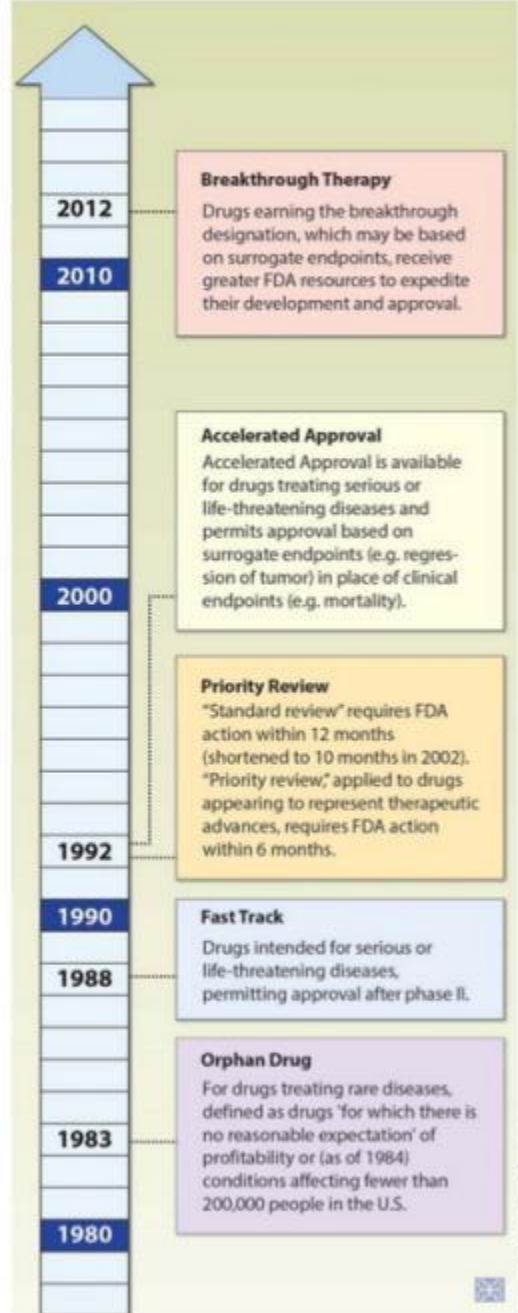
# Speed vs. Safety

## Five Existing Pathways

- Breakthrough Therapy
- Accelerated Approval
- Priority Review
- Fast Track
- Orphan Drug

Source: AS Kesselheim and JJ Darrow, "FDA Designations for Therapeutics and Their Impact on Drug Development and Regulatory Review Outcomes," *Clinical Pharmacology & Therapeutics* January 2015; 97(1): 29-36

Slide credit: Anthony D. So, MD, MPA, Program on Global Health and Technology Access, Sanford School of Public Policy, Duke University



**Figure 1** Timeline of formal enactment of special FDA designations for drugs. Description and chronology of five special regulatory designations for prescription drugs that can lead to expedited approval or accelerated testing.

# Current Reality: Faster Cures

- 56% of new chemical entities approved 2002-2013 benefited from at least one accelerated pathway.
- From 2001-2010 the FDA approved 63.7% and 85.7% of novel therapeutic agents earlier than did their European and Canadian drug regulators, respectively.

Source: AS Kesselheim and JJ Darrow, "FDA Designations for Therapeutics and Their Impact on Drug Development and Regulatory Review Outcomes," *Clinical Pharmacology & Therapeutics* January 2015; 97(1): 29-36



# Case Study: Bedaquiline (Sirturo)

- Approved based on a single, small, phase II randomized, placebo-controlled trial in patients with MDR-TB
- Surrogate endpoint: time until sputum free of TB
- standard combination of antibiotics for MDR-TB versus standard combo + bedaquiline.
- Drug killed more TB
- Drug also killed more patients (9 versus 2)
- Post-market study submission deadline– 03/2022

# FDA Commissioner Margaret Hamburg Remarks on 21<sup>st</sup> Century Cures Bill

“There’s a misperception that you might be able to speed up innovation by lowering the standards for safety and efficacy, and I think that would be a terrible mistake that would not only just damage patients, but industry as well.”

Source: Virgil Dickson, “FDA’s Hamburg, on her way out, blasts bill aimed speeding innovation,”  
*Modern Healthcare*, March 27, 2015.