Breakthrough Therapy Designation

May 12, 2014
Vital Transformation Webinar

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Friends of Cancer Research

FRIENDS of CANCER RESEARCH

A Unique Model to Advance Biomedical Research

- Each year Friends of Cancer Research (*Friends*) convenes conferences, forums and working groups, to address critical issues in the research, development and delivery of new drugs.

- These annual venues bring together leaders from **federal health and regulatory agencies, academic research centers, patient advocacy organizations and the private sector** to propose consensus solutions and develop a clear path forward on critical issues surrounding the development and regulation of drugs and therapies.

- Through our unique collaborative model, we have created a path to better drug development and approval through scientific, cultural, regulatory and legislative solutions.

Getting Breakthrough Therapies to Patients

- The 2011 Conference included a panel entitled: *Development Paths for New Drugs with Large Treatment Effects Seen Early*.
- Case studies: vemurafenib (*8xRR*) & crizotinib (*5xRR*)
- The panel proposed scientific strategies to ultimately expedite FDA approval for a drug showing dramatic responses in the early stages of development while maintaining drug safety and efficacy standards.

Goals of Breakthrough Therapy Designation

Goal 1: Expedite drug development process for products that show remarkable clinical activity early

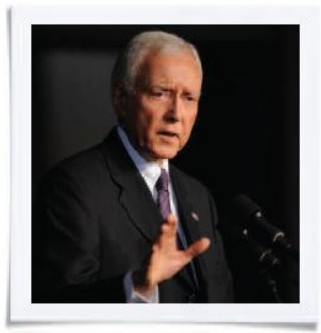
Goal 2: Minimize the number of patients exposed to a potentially less efficacious treatment

Getting Breakthrough Therapies to Patients

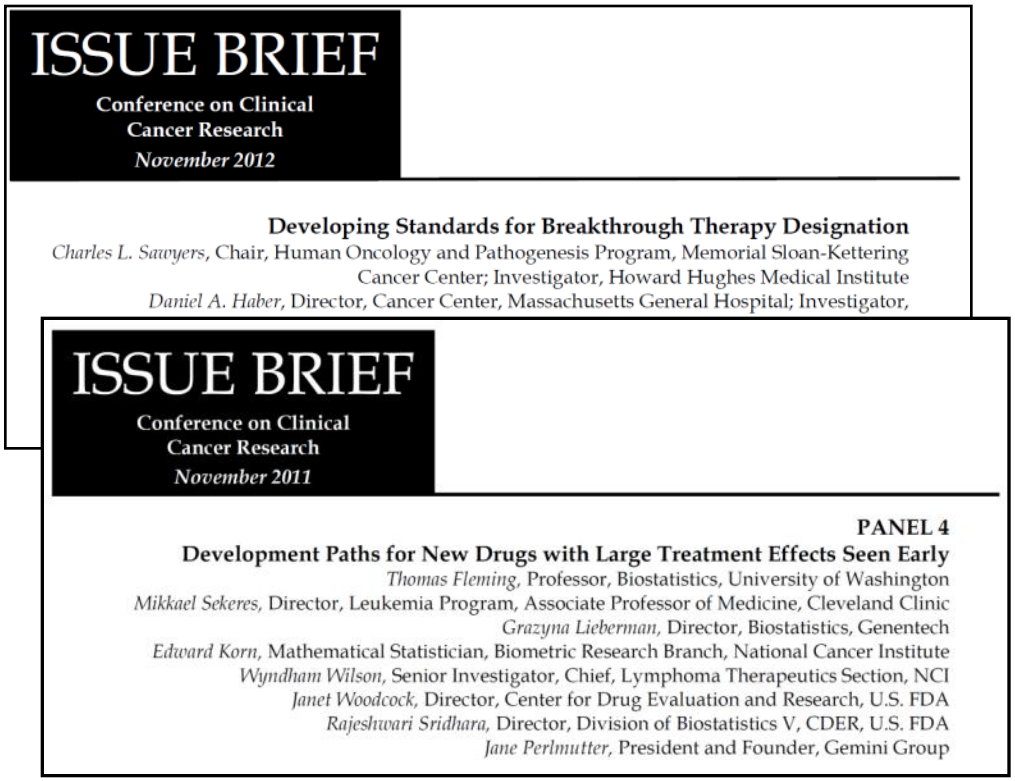
- On March 8, 2012, Friends of Cancer Research Exec. Director introduced Breakthrough Before House Energy and Commerce Committee

- On March 22, 2012, Friends of Cancer Research held a congressional briefing titled "Expediting New Treatments to Patients: FDA Approval Mechanisms"
  - "Breakthrough Therapy" designation received public endorsement from FDA, industry and academia.

- In the spring of 2012, the bipartisan *Advancing Breakthrough Therapies for Patients Act* was introduced by Senators Michael Bennet (D-CO), Orrin Hatch (R-UT) and Richard Burr (R-NC) and Representatives Diana DeGette (D-CO) and Brian Bilbray (R-CA).



Concept → Scientific Whitepaper → Bipartisan Legislative Solution → Tool in use by FDA to expedite the approval of multiple drugs in 13 months



ISSUE BRIEF

Conference on Clinical Cancer Research

November 2012

Developing Standards for Breakthrough Therapy Designation

Charles L. Sawyers, Chair, Human Oncology and Pathogenesis Program, Memorial Sloan-Kettering Cancer Center; Investigator, Howard Hughes Medical Institute

Daniel A. Haber, Director, Cancer Center, Massachusetts General Hospital; Investigator,

ISSUE BRIEF

Conference on Clinical Cancer Research

November 2011

PANEL 4

Development Paths for New Drugs with Large Treatment Effects Seen Early

Thomas Fleming, Professor, Biostatistics, University of Washington

Mikkael Sekeres, Director, Leukemia Program, Associate Professor of Medicine, Cleveland Clinic

Grazyna Lieberman, Director, Biostatistics, Genentech

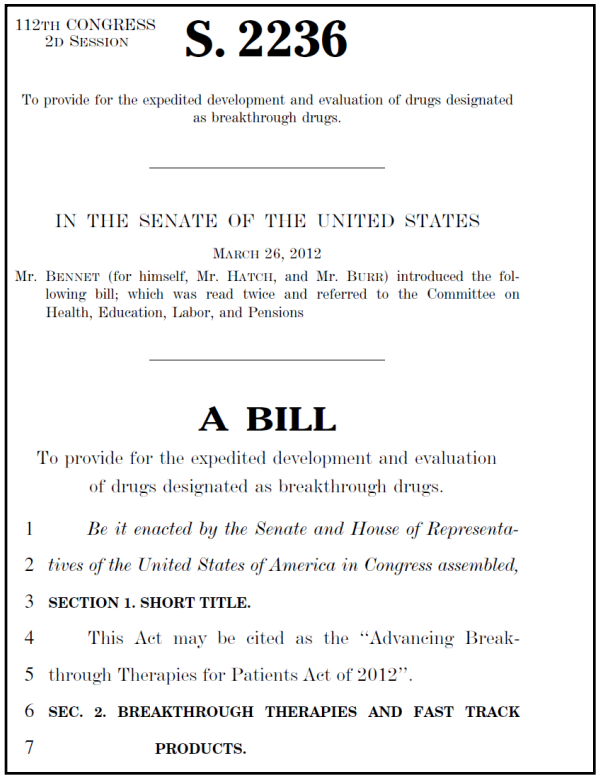
Edward Korn, Mathematical Statistician, Biometric Research Branch, National Cancer Institute

Wyndham Wilson, Senior Investigator, Chief, Lymphoma Therapeutics Section, NCI

Janet Woodcock, Director, Center for Drug Evaluation and Research, U.S. FDA

Rajeshwari Sridhara, Director, Division of Biostatistics V, CDER, U.S. FDA

Jane Perlmutter, President and Founder, Gemini Group



112TH CONGRESS
2D SESSION

S. 2236

To provide for the expedited development and evaluation of drugs designated as breakthrough drugs.

IN THE SENATE OF THE UNITED STATES

MARCH 26, 2012

Mr. BENNET (for himself, Mr. HATCH, and Mr. BURR) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To provide for the expedited development and evaluation of drugs designated as breakthrough drugs.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

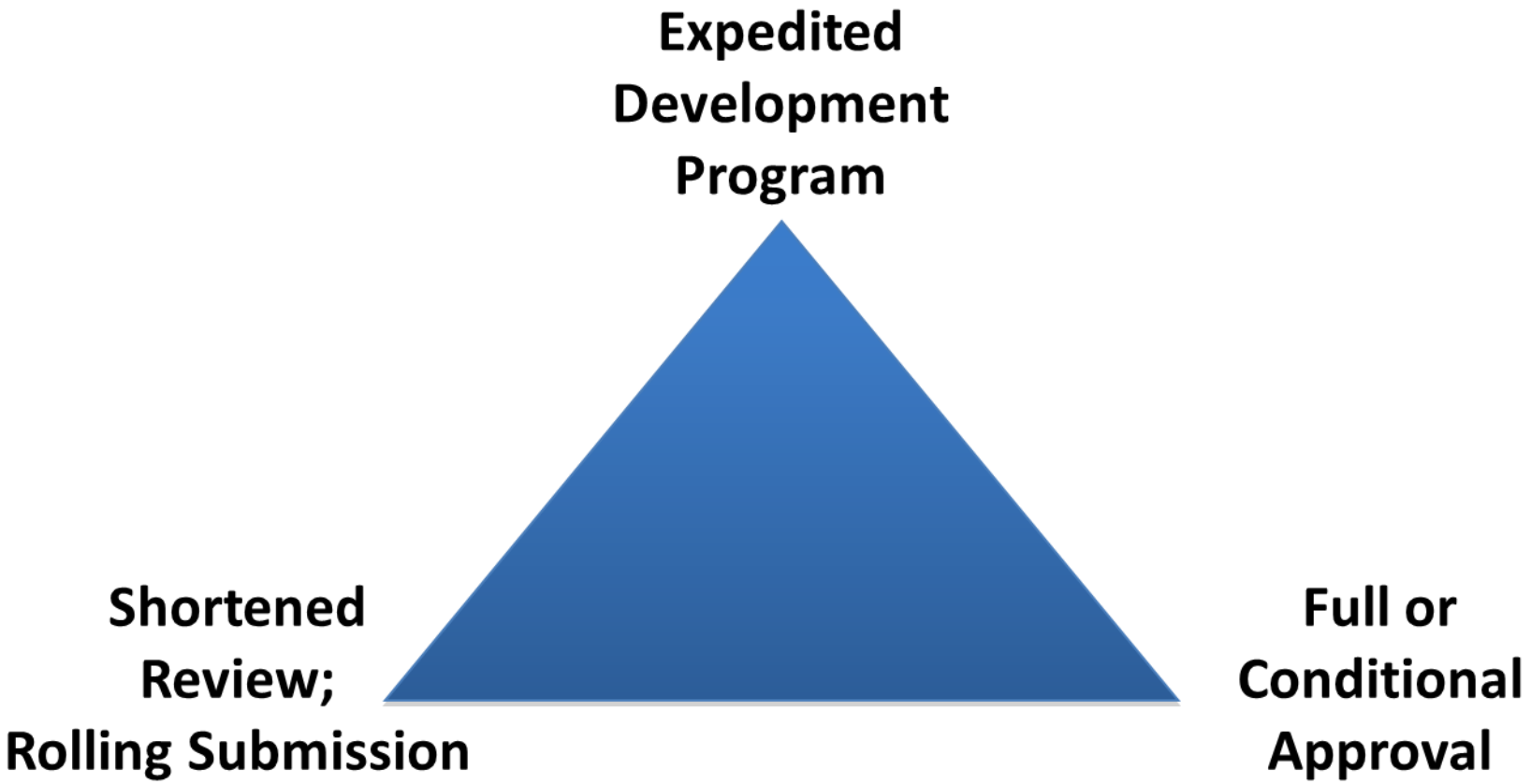
SECTION 1. SHORT TITLE.

This Act may be cited as the "Advancing Breakthrough Therapies for Patients Act of 2012".

SEC. 2. BREAKTHROUGH THERAPIES AND FAST TRACK PRODUCTS.

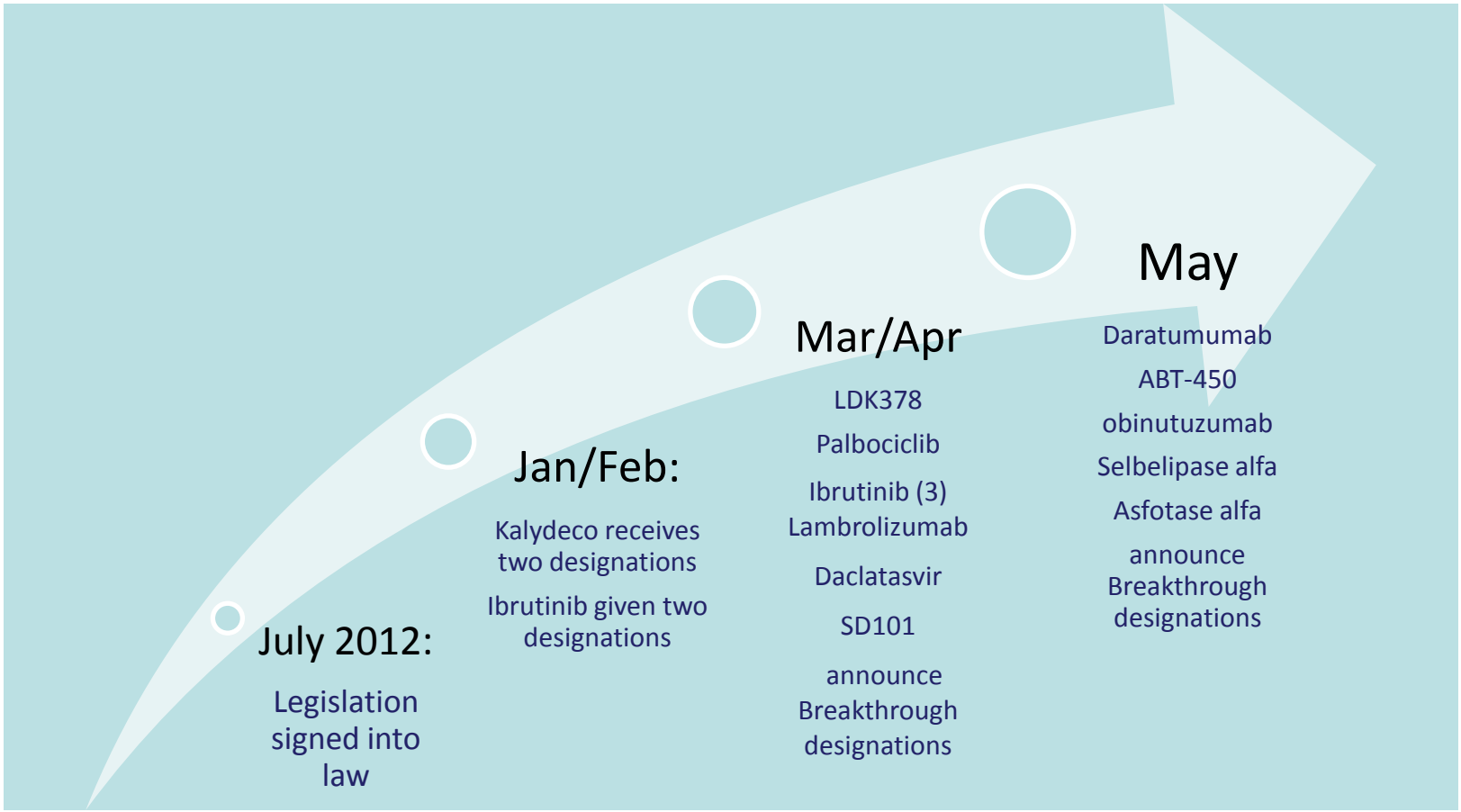
FRIENDS of CANCER RESEARCH

Distinguishing Features of Breakthrough Designation

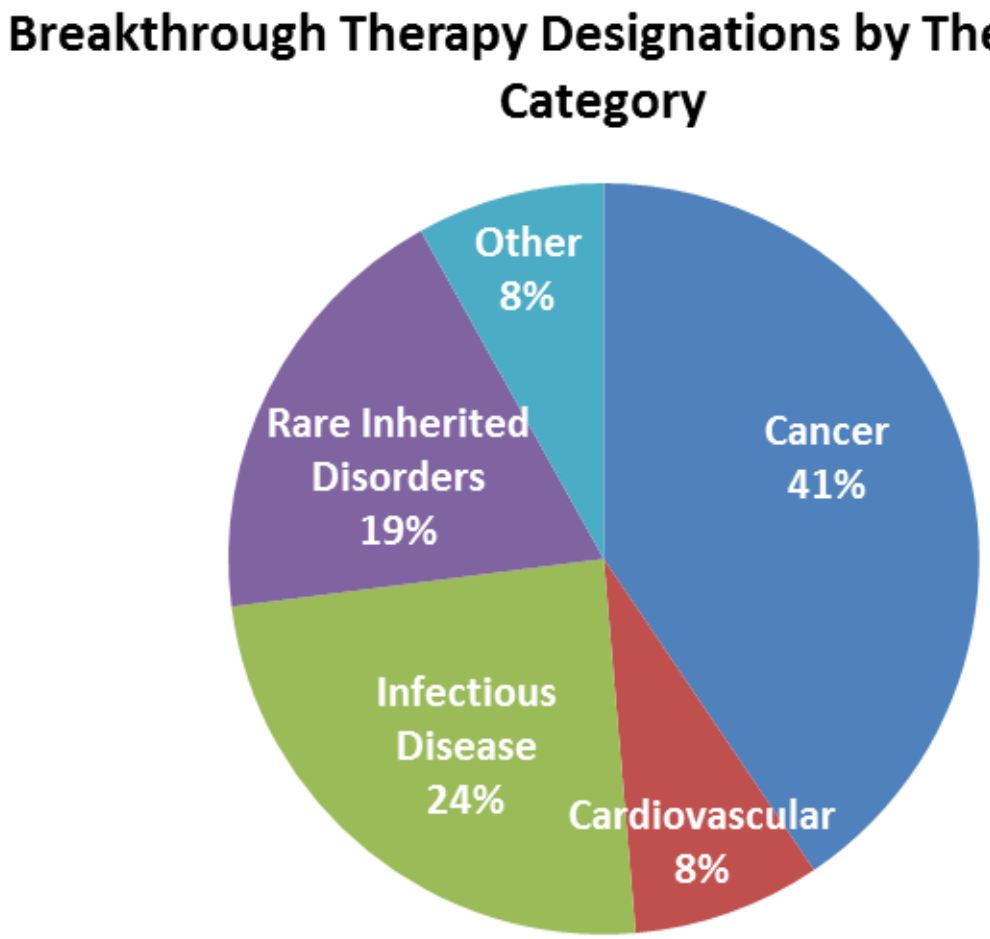


“All hands on deck” mindset – collaborative effort by senior FDA staff

Implementation of Breakthrough Designation



44 Breakthrough Designations Announced By Companies So Far



6 Drugs Approved through Breakthrough Program

Drug Name	Sponsor	Indication	PDUFA Deadline	Approval Date
Gazyva	Genentech	Chronic lymphocytic leukemia	12.20.13	11.1.13
Imbruvica	Pharmacyclics/J&J	Mantle cell lymphoma	2.28.14	11.13.13
Sovaldi	Gilead	Hepatitis C	12.8.13	12.6.13
Kalydeco*	Vertex	Cystic fibrosis	3.27.14	2.21.14
Arzerra*	Genmab/GlaxoSmithKline	Chronic lymphocytic leukemia	4.19.14	4.17.14
Zykadia	Novartis	Non-small cell lung cancer	8.24.14	4.29.14

Open Questions and Remaining Challenges

- What impact will Breakthrough Therapy Designation ultimately have on drug development times or approaches to clinical testing?
  - Imbruvica: went from first-in-man trials to commercial availability in 4.5 years.
  - Breakthrough not a guarantee of approval
- What impact will the designation have on uptake after approval?
  - Hefty price tags
  - Novel drug classes in pipeline- some agents with Breakthrough, others without
- Will this designation be too resource-intensive for FDA to keep up?
- Can manufacturing processes and development of companion diagnostics keep up with accelerated clinical development timelines?
  - Friends-Alexandria 2013 Breakthrough Diagnostics Conference: FDA review of companion diagnostics could prioritize “high-risk” data early in clinical testing and reserve “lower-risk” elements as needed for later stage or post-market evaluation
- How will this designation affect global drug development?

Role of Patient Community In Drug Development

- **Funding** - many disease-specific organizations provide critical funding for cutting-edge, translational science for near-term impact.
- **Advocacy –** external community must become advocates for a strong regulatory environment equipped with the resources and scientific expertise it needs to keep up with cutting edge technologies
- **Policy** - speak to what patients need to accelerate new tools and treatments to market and contribute scientific expertise to policymaking process
- **Partnership** - Public-private partnerships needed for goal-oriented collaboration.
  - Example: **Lung-MAP**: a multi-drug, multi-arm, biomarker-driven registration trial testing 5 compounds to treat squamous cell NSCLC as a result of extensive collaboration between Friends, NCI, Foundation for the NIH, FDA, lung cancer community, and the North American cooperative groups.