Regulatory Perspectives on Diabetes Drugs and Devices: JDRF Perspective

RICHARD A. INSEL, MD
CHIEF SCIENTIFIC OFFICER
JDRF
rinsel@JDRF.org

PRACTICAL WAYS TO ACHIEVE TARGETS IN DIABETES CARE
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JDRF Goals: Cure, Treat, and Prevent T1D

- **Cure**
  - To CURE T1D and remove the disease from the lives of our loved ones

- **Treat**
  - To develop better ways to TREAT T1D allowing people to live better lives

- **Prevent**
  - To PREVENT T1D from occurring & stopping its progression
JDRF: Accelerating the Discovery, Development & Delivery of T1D Therapies

Exploratory Research

Product Discovery

Product Development

Regulatory Approval

Health Insurance Coverage

Clinician Adoption
JDRF: Partners to Advance T1D Therapies
Regulatory Agencies Are a Key JDRF Partner

- Regulatory decisions affect much of the therapy pipeline
  - Affect time and resources for R&D starting with pre-clinical research

- For novel therapies, regulatory requirements for clinical endpoints and study designs often not yet defined, creating uncertainty
T1D Regulatory Challenges

T1D Therapies Scientific & Regulatory Challenges

- Staging of T1D
- Risk/Benefit
- Trial Design
- Biomarkers
JDRF: T1D Regulatory Science Initiative

- Staging and classification of T1D
  - At-risk/Prediabetes → T1D
  - Heterogeneity
- Patient perspective of risk/benefit
  - Longitudinal clinical outcome data
- Clinical trial design and strategy
  - Trials involving children
  - Prevention trials, Combination therapy trials
  - JDRF C-peptide database repository
- Biomarkers
  - Prognostic and Predictive
JDRF’s Regulatory Activities

- Dedicated full-time JDRF regulatory expertise for devices, drugs, and biologics (European JDRF staff)
- Co-hosting workshops with FDA, NIH
- JDRF-FDA one-on-one meetings, educational seminars
- Advocacy for class of agents vs. specific products
- Facilitate regulatory clarity, transparency, consistency
- Communicate unmet needs for T1D
JDRF Priority: Artificial Pancreas Device Systems

- Progress
  - Artificial pancreas systems: Priority for JDRF and FDA
  - 12+ JDRF-funded studies approved by FDA

- Challenges prior to 2011
  - Burdensome requirements
  - Delays
  - Unclear expectations
JDRF Worked with Clinical Leaders and FDA to Define Artificial Pancreas Device Systems Pathway

- JDRF 2010 clinical panel
- Guidance proposal based on clinical panel recommendations
- Endorsement of guidance by multiple clinical authorities including:
  - AACE
  - The Endocrine Society
  - ADA
  - AADE
Promising Results: Draft Guidance Released by FDA

- Provides a reasonable pathway for researchers and companies to follow
- Specific suggestions made by JDRF and clinical partners have been incorporated
- FDA seems open to comments on draft and is working to finalize it
- And – in March 2012 – FDA approved first outpatient academic study
JDRF’s Ultimate Goal

- Getting safe and effective new therapies that will prevent, treat, and ultimately cure T1D to the people who need them as quickly as possible
- Regulatory agencies play an important role in achieving this
- JDRF will continue to take a pro-active role in interfacing with regulatory agencies on behalf of its stakeholders