

# Translational Research Programs

Forum on Neuroscience and Nervous System  
Disorders

Board on Health Sciences Policy

Institute of Medicine

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Richard Insel, M.D.

Juvenile Diabetes Research Foundation



*Dedicated to finding a cure*

# JDRF

## Mission

*To find a cure for diabetes and its complications through the support of research*

## Strategy

*Proactively accelerate the discovery,  
development, and delivery  
of disease-modifying therapeutics to better treat  
and cure type 1 diabetes and its complications*



*Dedicated to finding a cure*

# Translational Research Gap

*Bridging the translational gap requires technical progress toward a drug candidate, procurement of necessary intellectual property, and **technology transfer to the commercial sector.***

## Applied Research

- Validated efficacy models and results
- Drug screening and optimization
- Preclinical toxicology package

## Successful Translation

## Transfer to Commercial Sector

- Components of intellectual property (IP)
  - Drug candidates
  - Drug targets
  - Process
- IP bundling

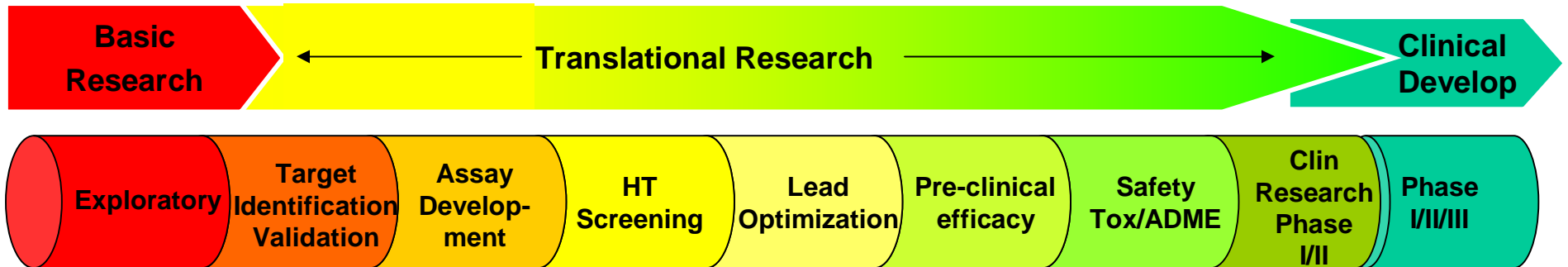
# Translational Issues: Lessons Learned

- Know your Product
- Know your Partners
- Know Yourself

# Know your Product

- Addresses significant unmet medical need
- Better than the current Standard of Care: Know the disease, treatments, and the landscape

# Translational Challenges: Scientific Validity and Technical Feasibility



§ Validated targets and pathways

§ Identify multiple druggable targets

§ Validated screening assays

§ Validated biomarkers: surrogate endpoints and susceptibility

§ Validated predictive animal models

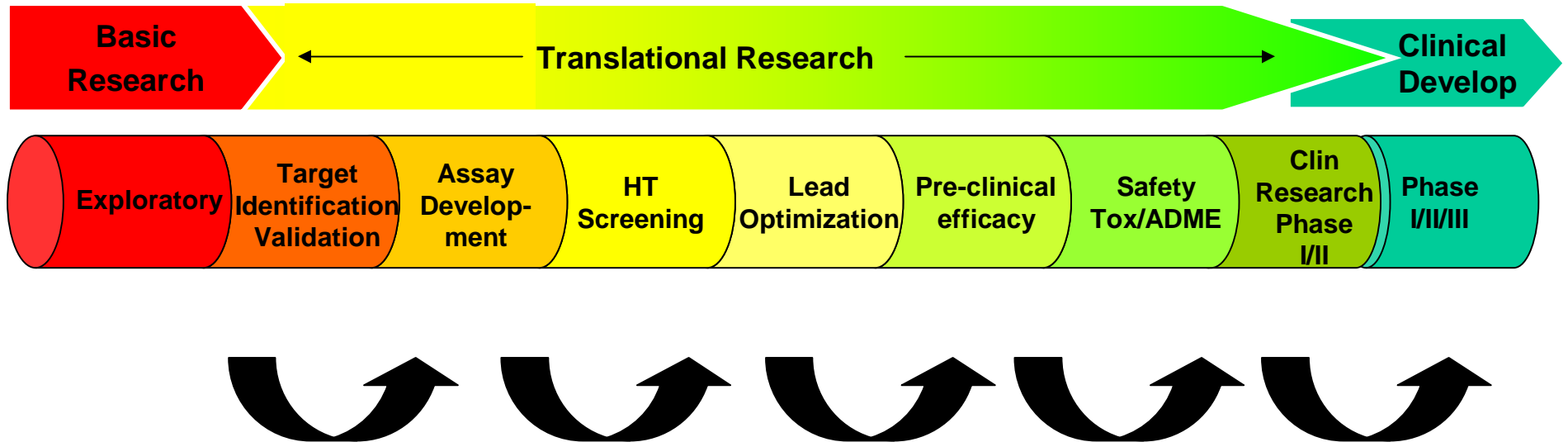
§ Early predictors of safety

§ Secured IP

§ Multiple Proof-of-Concept trials

§ Defined regulatory, reimbursement, and adoption pathways

# Translational Challenges

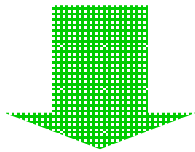


- Create a pipeline, not only a product
- Plan for success, anticipate failure
- Follow-on each stage efficiently with a line of site to a product

# Strategic Project/Product Evaluation Criteria

## Research Questions

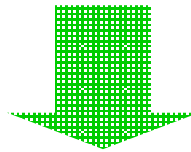
Scientifically valid?



- Scientific rationale
- Understanding of disease pathology
- Availability of druggable targets
- Target validation in vitro and in animal models
- Known molecular mechanism of action

## Development Questions

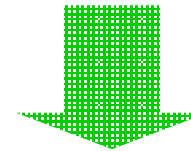
Technically feasible?



- Predictive animal models
- Manufacturing according to regulatory requirements
- Ability of trials to demonstrate efficacy, enable FDA approval
- Development length and complexity
- Trial cost

## Business Questions

Commercially viable?

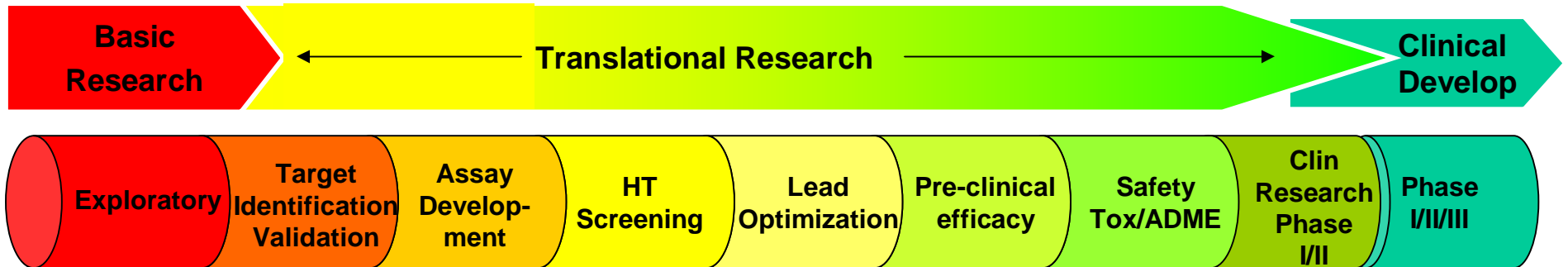


- Intellectual property status
- Manufacturing costs
- Market size/Patient population
- Unmet needs, opportunity
- Pricing flexibility
- Sales costs
- Competitive landscape

# Know your Partners

- Clarity on alignment of goals, motivation, expectations – Academia vs. Industry; Clinical Research vs. Clinical Development
- Do not estimate difficulty of changing a culture
- Engage commercial sector early
- Commercial sector: Expect financial constraints/stability issues

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§ Identify multiple targets

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# nature medicine

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<sup>16</sup> Bitter criticism sours new diabetes research plan

l. But off the record, many said that the program's level of oversight is unacceptable, and may be restrictive to the nonlinear manner of scientific discovery.

It's an interesting experiment," Keating says. "But it's very difficult to coerce academics to do this—it's like herding cats, really."

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# Know Yourself

- Clear on mission, role, niche,
- Focus
- Know risk tolerance
- Manage expectations-  
overpromise or hype, define  
success, metrics, educate your  
stakeholders

# Questions