Barriers to Translational Research in ACHD

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What is Translational Research?

“Translational research applies findings from basic science to enhance human health and well-being. In a medical research context, it aims to "translate" findings in fundamental research into medical practice and meaningful health outcomes.”
Goal:
Translating Science into High Quality Care

T0
Preclinical and Foundational Research
Informs research with humans

T1
Translation to Humans
Clinical interventions and their initial testing

T2
Translation to Clinical Settings
Inform evidence-based guidelines

T3
Translation to Practice
Implementation in real-world settings

T4
Translation to Populations
Population-based outcomes studies
The Bench to Bedside Abyss
Staff et al, Science Translational Medicine, 2014
Some general barriers…

- **Average time bench to bedside = 16-18 years**
- **Cost**
  - $4-5 billion for new drug development
- **Systemic barriers/bottlenecks**
  - “hand-off” between academia and industry
  - Lack of knowledge about the regulatory approval process among academic scientists
  - Transfer from the laboratory to “good manufacturing processes” (GMP)
“Innovators require greater clarity about regulatory pathways for innovative products and approaches, and innovators require greater consistency, efficiency, and communication with respect to their individual drug applications.”
What about ACHD?

- Heterogeneity of disease
- Lack of knowledge among scientists
- Lack of collaboration among ACHD providers and basic scientists
- Cost
- Sample size – single versus multicenter
- Patient participation
Strategies for Improvement

- Food and Drug Administration
  - Clinical trials, emerging technologies, harnessing “large datasets”
  - Training seminars for clinical investigators
  - Investigational Device Exemption process

- Funding
  - Most powerful tool for influencing research
  - Goal: to have a translational focus
  - Clinical and Translational Science Awards
Strategies for Improvement

- Understanding of regulatory agencies
  - Center for Medicaid Services
  - Food Drug Administration

- Investigator focus
  - Increased collaboration
    - Investigators/clinicians/funding and regulatory agencies
  - Learn the process for translational research
  - ”Translational Integrator” – facilitates collaborations noted above
Strategies for improvement in ACHD

- Improve partnerships with funding agencies
  - Mission of FDA and NHLBI to work with patients
- Build relationships with FDA and CMS
- Multicenter research network
- Partnership with advocacy organizations
  - Advertising studies
  - Patient recruitment
  - Study design
Strategies for improvement in ACHD

- Team science
- Build from experience from other fields
  - Childhood Cancer Survivor Study
- Improve collaboration with basic scientists
  - Vascular biology
  - Thrombosis research
  - Immunology
  - Neuroscience
Conclusions

- Complex process that takes time
- Involve patients in the process
- Take a seat at the table
- Advocacy
- Collaboration, collaboration, collaboration!
Thank you!