Infantile Spasms Initiative: Directed Team Science

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• January 2012 – Key opinion leader (KOL) summit in San Francisco to identify one or more research “opportunity areas”

• Outcomes of that meeting
  – Infantile spasms identified as focus area which could deliver a breakthrough in the “short term”
  – Take a different approach – create a “team” of investigators working toward a common goal, utilizing their different areas of expertise
  – KOLs agreed to become the advisory panel for the initiative
• Why infantile spasms?
  – Seizures are stereotypical, thus making diagnosis more clear compared to other seizure types
  – Prognosis is particularly poor and frequently results in life-long developmental disability (need for better therapies)
  – Atypical EEG serves as a predictive biomarker that can be used to track success of therapy
  – Animal models are available that can be used for the early identification of new therapies; e.g. anti-inflammatory drugs
  – Success can be measured in the short term; years vs. decades
FIGURE 1: Number of New Consortia Launched, by Year

1 1 3 2 3 4 5 7 9 3 9 17 21 19 29 30 43 51
“agreement on a mission that addresses a shared need with a strategic and milestone-driven plan to achieve output that, in turn, can be broadly used by each stakeholder”

“an integrated research plan that leverages the research resources and knowledge from each stakeholder”
April 2012 - Request for proposals (RFP) circulated
  - Key differences from CURE’s traditional programs: milestone-driven, no budget limits (each project’s budget would be evaluated individually, on a milestone-by-milestone basis)

Program goal: To support collaborative, milestone-driven efforts that advance the understanding of the underlying pathology of infantile spasms and lead to the development of a disease-modifying therapy or cure for infantile spasms.
June 2012 - Received 27 letters of intent (LOIs)

August 2012 - Invited 12 full proposals

October 2012 – Received 11 full proposals for review

November 2012 - Selected 8 proposals to support
The Team

- Chris Dulla, PhD – Tufts University
- Aristeia Galanopoulou, MD, PhD – Albert Einstein College of Medicine
- Jeff Noebels, MD, PhD – Baylor College of Medicine
- John Swann, PhD – Baylor College of Medicine
- Libor Velisek, MD, PhD – New York Medical College
- Manisha Patel, PhD – University of Colorado Denver
- Doug Nordli, MD – Lurie Children’s Hospital
- Elliott Sherr, MD, PhD – University of California, San Francisco

Animal models, mechanisms

Tool development

Human, clinical studies
January 2013 – brought 8 lead PIs and advisory panel together in Washington, DC
  – Each PI presented his/her proposal to the team for feedback
  – The group decided which aim should be the first milestone
  – Advisory panel met with CURE representatives on second day to discuss budget for each year 1 milestone
• Quarterly GoToMeeting teleconferences – each PI presents progress to the group for feedback

• Face-to-face meetings to continue at least once per year
  – 3 to date: January 2013, December 2013, July 2014
Progress

- 15 months since funding began
  - $2.1 million committed, to date

- 2 promising compounds
  - Currently testing in all models across 5 laboratories

- July 2014 – workshop to discuss results, think through steps to get to clinical trial, if applicable
• It’s hard to truly work as a team!

• Composition of the team is crucial

• Stay tuned....