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Infantile Spasms Initiative: Directed Team Science

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The Idea

- 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

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Rationale for IS

- 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

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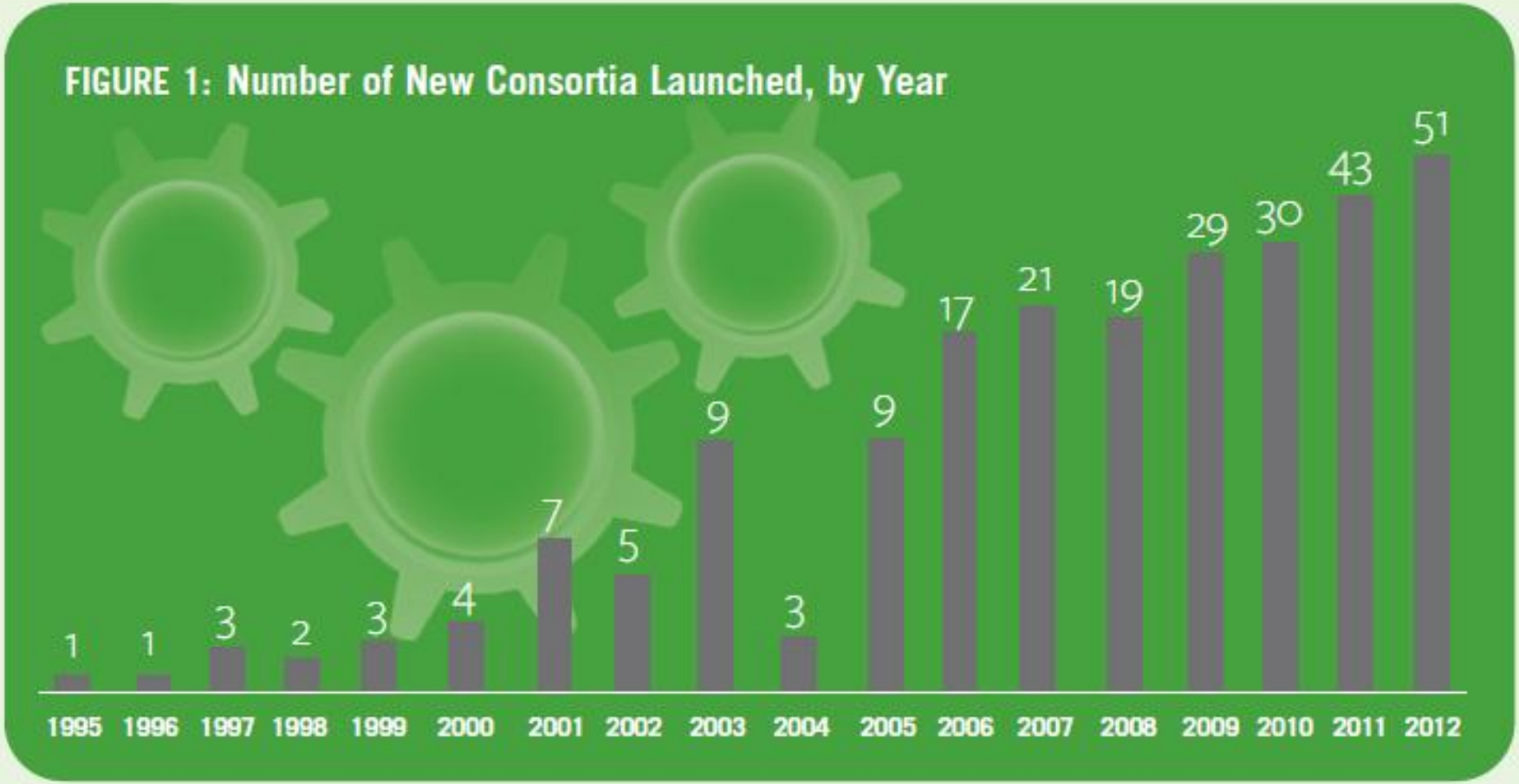
Research by Consortia



www.fastercures.org/consortiapedia

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Research by Consortia

- "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"

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Enacting the Vision

- 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.

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Timeline

- 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

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The Team

- Chris Dulla, PhD – Tufts University
- Aristea 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

Animal models, mechanisms

- Manisha Patel, PhD – University of Colorado Denver

Tool development

- Doug Nordli, MD – Lurie Children's Hospital
- Elliott Sherr, MD, PhD – University of California, San Francisco

Human, clinical studies

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Process

- 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

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Process

- 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

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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

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Lessons Learned

- It’s hard to truly work as a team!
- Composition of the team is crucial
- Stay tuned….