

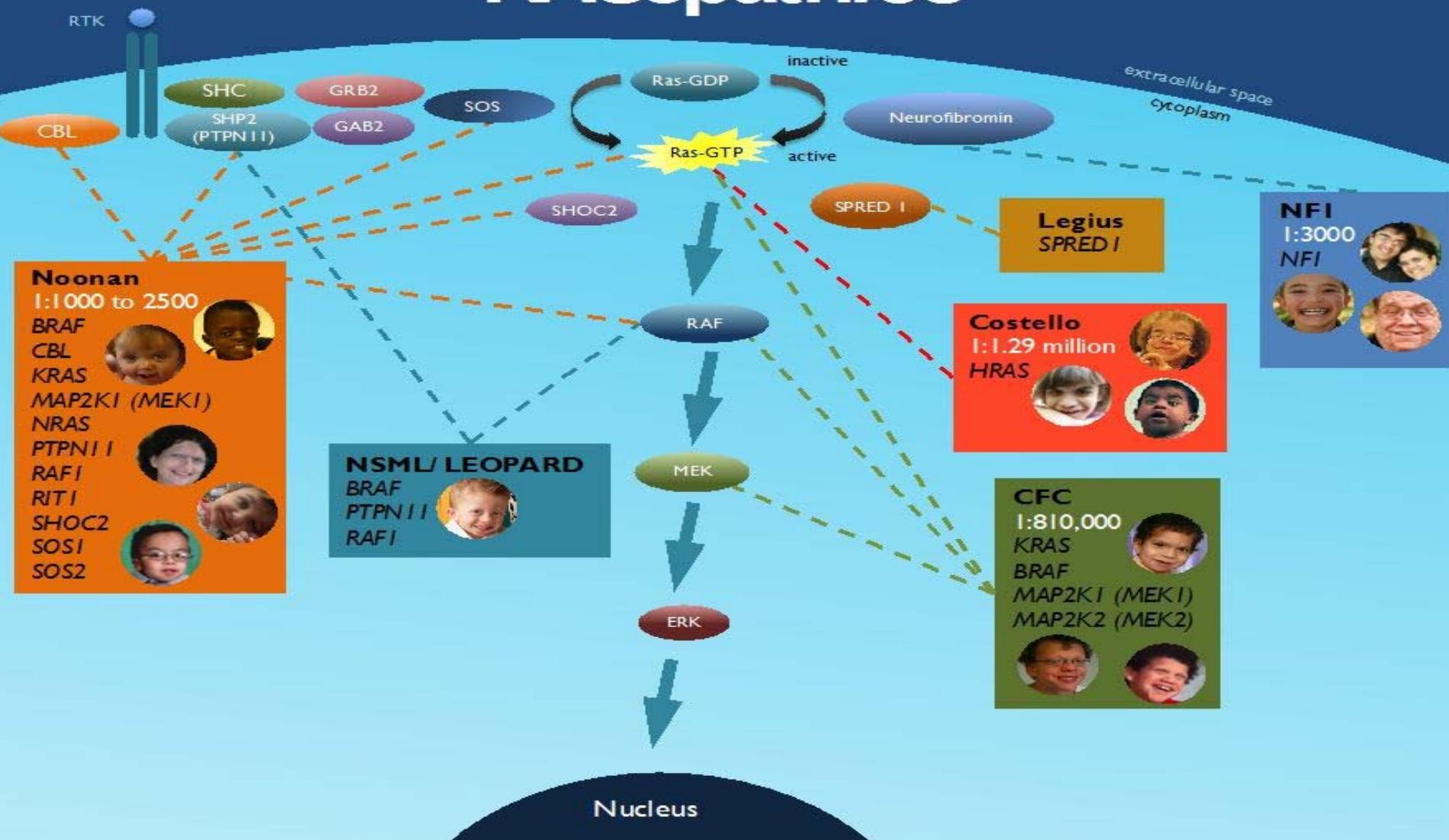


Lisa Schill
Vice President RASopathies Network USA
Parent Advocate
lschill@rasopathiesnet.org
<http://rasopathiesnet.org>

My Journey into the Rare Disease World



RASopathies





Our mission is to advance research of the RASopathies

Did you know??

11-14 years to develop a new treatment!!

Up to 2.6 BILLION dollars!!

The Drug Development & Approval Process. (2016). http://www.fda.gov/03_drug_development.php

Mullin, Rick.(2014, November 24). <http://www.scientificamerican.com/article/cost-to-develop-new-pharmaceutical-drug-now-exceeds-2-5b/>

That is A LOT of Bake Sales!!



Drive LEGISLATION to Create Change
&
CURE THE PROCESS



Rare Disease Legislative Advocates Legislative Conference



WANTED



CURES New Jersey Roundtable



21st Century Cures Roundtable
August 4, 2014 • 10:00AM
Celgene • Summit, NJ

Agenda:

- Introduction & Welcome Remarks – Rich Bagger, Senior Vice President, Corporate Affairs and Strategic Market Access, Celgene
- Introduction of 21st Century Cures Initiative – Rep. Leonard Lance
- Panelist Introductions
- Moderated Discussion
- Audience Q&A

Panelists:

- Mark Alles
COO and President, Celgene
- Ricki Fairly
President & Thought Leader, DOVE Marketing
- Scott Mellis, M.D., Ph.D.
Vice President, Translational Medicine, Regeneron
- Dr. Fernando Muzzio
Distinguished Professor, Chemical and Biochemical Engineering, Rutgers University
- Dr. Paul Reider
Pharmaceutical Specialist and Lecturer, Princeton University
- Brian Rosen
Leukemia & Lymphoma Society
- John E. Runnells
General Partner, The Vertical Group
- Debra L. Wentz, Ph.D.
CEO, New Jersey Association of Mental Health and Addiction Agencies





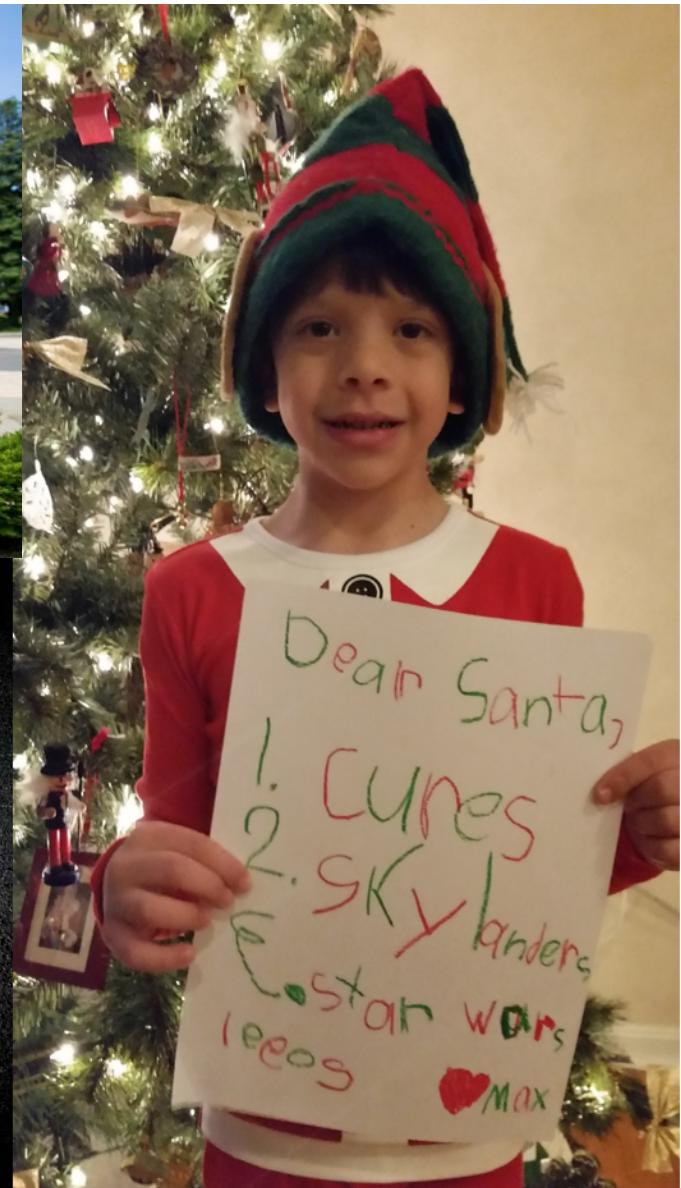
21st Century Cures Initiative

- Bipartisan Bill
- The Committee spent over 1 year gathering input from stakeholders and released 4 draft bills



21st Century Cures/H.R. 6

- Rare Disease Community Priorities:
 - Orphan Product Extensions Now, Accelerating Cures & Treatments (OPEN ACT)
 - Improve biomarker qualification
 - Improve FDA's ability to recruit & retain staff & keep up on the latest science
 - Billions in NIH & hundreds of millions in FDA Funding
 - Expanding Hope Act (Priority Review Vouchers)
 - Neurological Disease Surveillance
 - Compassionate Use Reform & Enhancement Act
 - Patient Focused Drug Development



21st Century Cures in Committee

51-0 Vote





21st Century Cures Floor Vote



Onto the Senate!



