Newborn Screening Initiative: CALIFORNIA

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Newborn Screening: Health Implications

- The average rare disease patient goes 7 years undiagnosed
- A treatable, progressive disease can become a death sentence or result in a lifetime of debilitation
- Newborn screening can detect diseases early enough to slow, and in some cases halt disease progression
Sibling Study of MPS VI

Sib 2
3.6 yrs old
Treatment From Birth

Sib 1
7 yrs old
Treatment from 3 yrs old
Improved Spine If Treated Early at Birth
3 years of treatment from age 3 or birth

3 y.o. Sister

Newborn Brother
Adrenoleukodystrophy (ALD)

Early Diagnosis

Samuel was diagnosed with ALD at age 5 and received early treatment. He plans to become a doctor when he grows up.

Late Diagnosis

Jonathan suffered severe brain damage before he was finally diagnosed with ALD at age 7. He is now permanently disabled.
How does a new disease or screen get added in states?
- Lobbying from industry or patients groups
- However, there are 50 states and 7,000 rare diseases
- Places undue burden on small patient orgs to get their disease screened for in all states
- Legislative delays can result in babies falling through the cracks and the legislative process can be painfully slow
- Outcome is that many more kids will go undiagnosed and without proper treatment
Newborn Screening is variable

Screening of the 32 Core Disorders

*Screening is on the state panel and fully implemented in the state

No Disease Is Too Rare to Deserve Treatment
An improved landscape

- What if there was a committee that used science to determine which diseases to screen for instead of politics?
Advisory Committee on Heritable Disorders in Newborns and Children

Advisory Committee on Heritable Disorders in Newborns and Children

2016 Meetings

- February 11-12, 2016
- May 9-10, 2016
- July 25-26, 2016

The Advisory Committee on Heritable Disorders in Newborns and Children (Committee) was established under the Public Health Service Act, Title XI, § 1109 (42 U.S.C. 300b-10), as amended by the Newborn Screening Saves Lives Reauthorization Act of 2014 (P.L. 113-240).
What the RUSP Provides

- Generate a list of disorders that they recommend be screened for
- Current recommend ~32 core disorders and 26 secondary
- They will only recommend diseases that have an existing treatment
A Policy Solution

- A new approach that requires diseases recommended at the federal level to be screened for in the states
- Running legislation in California
- If passed, it would require screening of MPS 1 and Pompe disease in CA
- Alleviate the need to run a new bill for each disease
Our Lobbyists – Mike Carpenter & Mike Knudsen

- Carpenter represents California Life Science Association (CLSA)
- Knudsen played a vital role in passing ALD legislation in CA, requiring the state screen for the disease once added to the RUSP.
Strategy and Timeline

- January: Create draft language
- February: Meet with Sponsor
- February 19th: Introduced Legislation
- March: Hold community webinar and circulate organizational sign-on letter
- May-July: Whip co-sponsors, hold briefing and conduct meetings
Launching the bill – SB 1095:
Dr. Emil Kakkis, EveryLife President
Dr. Richard Pan, California State Senate
CA Legislative Deadlines 2016

- **Sept. 30** Last day for Governor to sign or veto bills passed by the Legislature
- **Oct. 2** Bills enacted on or before this date take effect January 1, 2017 (at the end of 2 year cycle)
- Plan B – if bill fails to pass in 2016, will have to reintroduce in 2017, wait until end of next session in 2018, with enactment in 2019!
Ways to engage on Newborn Screening

- Sign-on letter
- Join Community Congress
- We Need ECONOMIC data and case studies
- California Advocates: WE NEED YOU!
February 26, 2016

Dr. Richard Pan
State Capitol, Room 4070
Sacramento, CA 95814

Dear Dr. Pan,

As national patient organizations, we write today to thank and commend you for your very important leadership on newborn screening that is embodied in SB 1095. Newborn screening has proven to be essential to the long term health and well-being of babies born in California and across the United States. SB 1095 provides a much needed, practical, science-based approach to improving public health for all of California’s children. Every year, millions of babies born in the U.S. are screened for a variety of diseases and conditions that might otherwise go undetected. These simple screens help provide lifesaving early warnings of conditions. This allows for the earliest diagnosis and access to potentially life-saving and life-altering treatments for babies. Early treatment is vital in ensuring the best possible life-altering health outcomes for children. In many cases, early detection can avert costly and risky medical procedures later in life. A study in the journal Pediatrics found that California saves $9.32 in health care costs for every dollar spent on newborn screening.

Under current practice, states take many different approaches to newborn screening. This patchwork approach creates wide variation in disease detection and access to treatment. Currently in California, if patients and their families seek to expand screening, they must go through the expensive, exhausting, and uncertain legislative process. This places an incredible burden on small, resource-limited organizations and families who seek to update state law. California has long been a leader in this area, but its current approach to reviewing and approving screening for additional diseases has created a lag behind current medical recommendations. Every day of delay in testing is another day of heartache for patients and their families that could have been avoided with more timely action.
Community Congress

- Strategic advisory body for the EveryLife Foundation
- Three working groups comprised of patient organizations and industry representatives
- Newborn Screening Co-Chairs:
  - Kristine Caalim, Zeqing for a Cure
  - Jay Greissing, Shire
- Membership is free for patient orgs, visit:
- www.RareCongress.org to sign-up
We Need Economic Data

EArly Diagnosis for ALD is both Humane & Cost-effective

Keith Van Haren MD
Department of Neurology

March 21, 2014
Looking Ahead

- Create tool kits for introducing similar legislation in other states
- Would like to introduce legislation in two new states in 2017
- Host informational workshops for state legislators and public health departments on NBS & genetic diseases
Questions & Discussion

- Thank you to our sponsors!
- mbronstein@everylifefoundation.org