

Opportunities and Engagement with Newborn Screening Programs



A small heel prick can save a life!

Everything I **NEVER** thought I would have to know about Rare Diseases

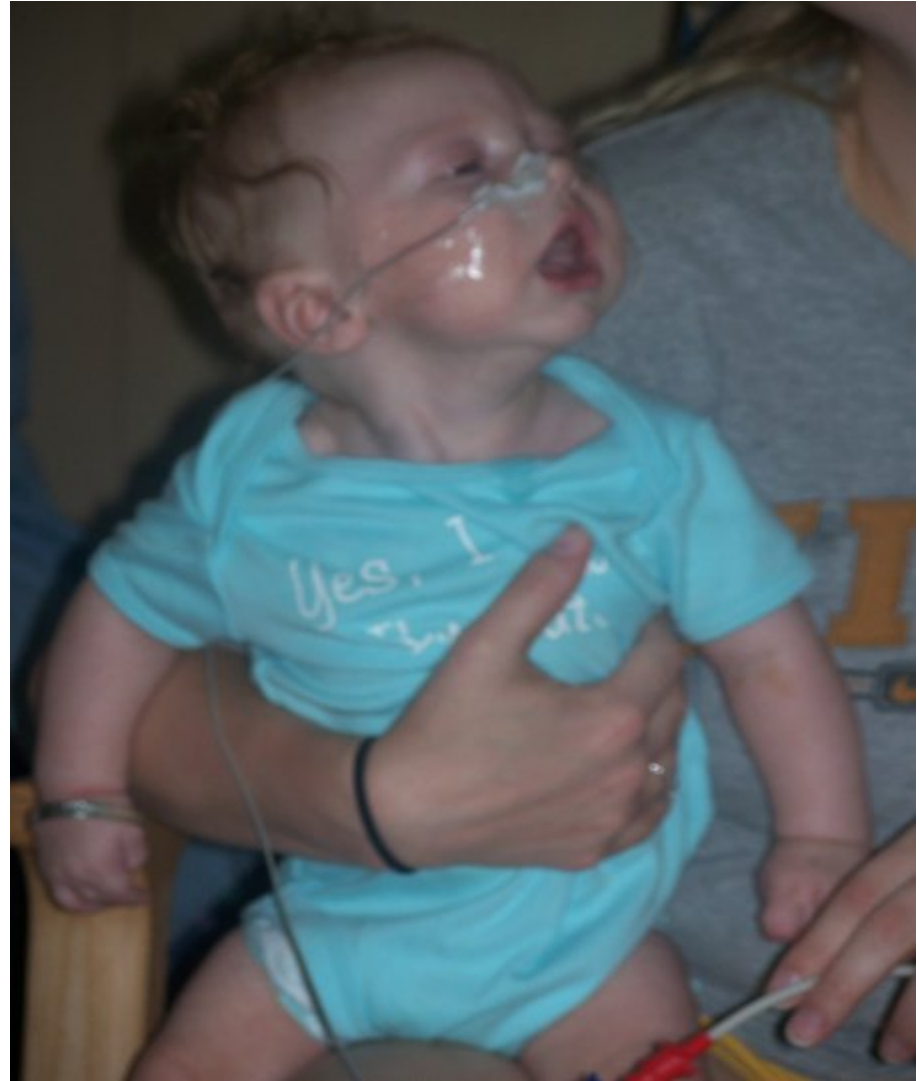
- My Missouri Newborn Screening Journey
- Just a Grandma 2008



Brady Alan Cunningham 4/16/2008-4/06/2009

Missouri Newborn Screening Journey

- The Diagnosis—
From acid reflux to
KRABBE DISEASE
- Research
- Advocacy
- Legislative Process
- 2009 Brady's Law
PASSED
- Hiccups and
Resolutions



But...

- **What about the RUSP???**

Recommended Uniform Screening Panel

- Should states screen for diseases not on the RUSP?
- Delay in NBS
- Health/Life of the child and family
- Should 100% Cure be necessary for RUSP?

NOT Added to the RUSP

- ***Krabbe Disease (2009-2010)***
External Evidence Review
- [Executive Summary](#) (PDF - 23 KB)
- [Summary](#) (PDF - 543 KB)
- [External Evidence Review Report](#) (PDF - 51 pages)
- [External Evidence Review Letter](#) (PDF - 1 page)
- [Letter from Committee](#) (PDF - 2 pages)
- ***Fabry Disease (2008)***
- [Letter from Committee](#) (PDF - 1 page)
- ***Pompe Disease (2008)***
- [External Evidence Review Report](#) (PDF - 28 pages)
- [Letter from Committee](#) (PDF - 2 pages)

- <https://www.hrsa.gov/advisory-committees/heritable-disorders/rusp/previous-nominations.html>

SAVED Because of NBS





Ezra has Krabbe Disease detected through NBS in MO and living a healthy and happy life with his parents in St. Louis, MO. He will be 3 years old next month!
Yet...Krabbe is not on the RUSP

Continuing to Expand NBS in MO

- May 2016 SCID Legislation Passed
- December 2016 Filed HB 66 SMA & MPS II
- SB 50 signed into Law July 20, 2017
- 2019 Missouri is screening for SMA & MPS II
- Site Visits from around US, the world, and CDC continue in MO

Where Does the Advocate Start?

- Research

What does your state currently screen for?

How is NBS funded in your state?

How many live birth are there in your state?

- Your Legislator (Rep or Senator)

- State Health Lab

- Genetics Advisory Committee

- Other Groups

Motivation/Roadblocks

- What are the benefits of screening?
- Why do we need legislation?
- Who will this affect? Put a face to the dollar signs!
- Make it personal
- Palliative Care vs Screening Costs
- You are MORE POWERFUL than you know!

Advocacy for NBS and the role of a Healthcare Provider

- Advocates and Providers need to work together
- Build relationships
- Search out the experts
- Knock down the barriers to communication
- Continue Research

Next Steps

- Get Legislation Filed
- Get Media Involved—TELL YOUR STORY
- Get on Social Media

Use your Sphere of Influence

- Get Prepared to Testify in front of Committees
- Get Determined & Stay Involved
- DON'T TAKE NO FOR AN ANSWER!
- Get the Bill Passed and Signed!

Thank you to Brady, Our Hero!



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