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| Meeting Minutes  Chloe Barnes Advisory Council on Rare Diseases  April 28,2022  3-4:30 |

# Call to order

The quarterly meeting of **the Chloe Barnes Advisory Council on Rare Diseases was** called to order on Thursday, April 28th at 3pm by Dean Tolar. Those present:

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| Member | Present | excused |
| Abigail Miller | x |  |
| Rep Kelly Morrison |  | x |
| Amy Gaviglio | x |  |
| Arthur Beisang | x |  |
| Barbara Joers | x |  |
| Jackie Foster | x |  |
| Angela Cowen | x |  |
| Karl Nelsen | x |  |
| Maureen Alderman | x |  |
| Kerry Hansen | x |  |
| KrisAnn Schultz | x |  |
| Dawn Stenstrom | x |  |
| Lisa Schimmenti |  | x |
| Nicole Brown |  | x |
| Paul Orchard | x |  |
| Rae Blaylark | x |  |
| Sen. Julia Coleman |  | x |
| Sen. Matt klein |  | x |
| Sheldon Berkowitz | x |  |
| Soraya Beiraghi | x |  |
| Dr. David Tilstra | x |  |
| Thomas Blissenbach | x |  |
| Timothy Schacker |  | x |
| Rep Tony Albright |  | x |

# Approval of minutes

The legislative session has begun. The bill to move the Council to the legislature is being reviewed. This will be discussed more later in the agenda. Following the recognition of Dr. Paul Orchard as a healthcare hero by St. Paul Business Journal, Dean Tolar entertained a motion to approve the minutes. Dr. Orchard made motion, Karl Nelsen seconded and motion approved.

# New Member Introductions:

* Angela Cowen
  + LICSW (Social Worker)
  + She worked at a private practice
  + Her experience with rare diseases is in a personal capacity
* Dawn Stenstrom
  + Representing the Industry Seek
  + Work in regulatory affairs with Boston Scientific
  + She has a lot of experience with medical devices and clinical trials
  + Is a rare disease patient and very grateful for this work
* Moreen Alderman
  + Patient and Caregiver Representative
  + Works at General Mills in Food and Safety Quality
  + Her daughter had a newborn screening and was diagnosed with ASA

# Mission Moment

* Speaker
  + Josh Berkowitz
    - Vice President of Finance at Hillel
    - Patient with VACTERL Syndrome
* Summary
  + Talks about his experience has a rare disease patient and the difficulties he faced (i.e. isolation from other children, multiple surgeries, difficulty breathing, and a stroke).
  + Talks about a show he put on to entertain and educate audiences
  + His goal is to make people laugh and continue to enjoy his life.

# NORD Annual State Report Card

* Speaker Annissa Reed, Regional Policy Director for the Eastern Region for NORD presented on the NORD state report card whose purpose is to determine state performance on various policies. Following presentation Council member asked questions and proposed ideas for using the report card to address needs in Minnesota

# Newborn Screening Taskforce Presentation

* Speaker: Erica Barnes presented on activities on behalf of the work consisting of: Dr. Beisang, Nicole Brown, Amy Gaviglio, Paul Orchard
* Summary
  + Overview, Council Mandate
    - The council is charged with consulting and cooperating with the Department of Health, the advisory committee on heritable and congenital disorders.
    - Ways that engagement with HBS relates to the council's mission and goals
      * insurers early diagnosis of rare diseases, many of which are otherwise virtually impossible to diagnose in time for intervention
      * fosters coordinated care through follow-up programs, clinician educational support
  + Two Approaches to Engagement
    - Proactive
      * NBS pilot projects
      * guest speakers
      * position papers
    - Responsive
      * patient community education
      * identification of experts in specific rare diseases
      * participation on evidence review panels
  + Questions Posed
    - Proactive: how proactive versus responsive should the council be?
      * Council should be a catalyst for forward thinking. NBS committee has a defined scope largely related two disease addition, so council should partner to foster innovation
    - Community requests: what process should we follow in response to a requested letter of support from a patient community? What if a patient community wants to pursue strategies to add conditions to NBS outside of the current process?
      * Established process is best practice, we do not support going outside to find procedure RUSP addition is ideal but not necessary
    - Innovation: Are there bigger picture forward thinking NBS initiatives that the council can be championing?
      * The current state of newborn screening in the US will be significantly impacted by the increased ability of gene therapies, advances in genetic testing capabilities. There's an opportunity for Minnesota to be the leader
  + Topics for Further Exploration
    - Looking into new ways to identify disease is better and faster. Incorporating Next Generation Sequencing into state screening programs
    - Ensuring sustainability for NBS to keep up with future demand
    - Pilot projects
      * Opt-in screening projects
      * ScreenPlus, NY
  + Possible Deliberation Process
    - Priority topic identification
    - Host speakers and literature reviews
    - Convene roundtable
    - From position paper from output
    - Communicate recommendations to relevant stakeholders
  + Questions
    - Is Heritable Screening different than NBS?
      * Yes, and we are looking to collaborate. HSD wants to define and find new rare diseases.
    - Does the taskforce consider cost?
      * If it is a part of NBS it should be covered. We will touch base with Screen Plus and see.
    - Is NBS only diseases rapid diagnosis can lead to improvements in care and where treatments are available?
      * Some of the new conditions push rapid diagnosis a bit, but yes to treatments available.
      * Keep in mind we may be identify any diseases, but if there is no treatment should it be added to NBS?
    - How are we ensuring appropriate risk and benefits of families?
      * This is why not many people do this. We have not solved how to do it right and sustainably

# Public Comment Submission

* What is a Medicaid demonstration waiver?
  + Waiver to implement experimental, pilot, or demonstration projects that are found by the secretary to be likely to assist in promoting the objectives of Medicaid program
  + It gives states additional flexibility to develop state specific policy approaches
  + it's usually approved for an initial 5 year. And can be extended for an additional three to five years
* Rationale for public comment submission
  + Specialist and health systems in Minnesota see individuals from across the US. This has the potential to impact access for our patients.
  + if Oregon is successful other states could introduce similar proposals
* Comment content
  + unique characteristics of the rare disease community
  + necessity for alternative approval pathways that match the characteristics of the rare disease community
  + alternative pathways are not inferior pathways
  + excluding accelerated approval treatments is an ineffective cost control method
  + limiting access to lifesaving treatments for patient populations with unmet medical needs contributes to disparities
* Questions/Comments raised
  + Amy Gaviglio we wanted to educate the state to proactively address the concerns articulated in the response to the Oregon waiver
  + Sheldon Berkowitz questioned Will this actually happen? Oregon has a history of making decisions by the state. MN doesn’t have that history. Amy Gaviglio responded that the environment is more volatile
  + Council member asked Is NORD tracking this?
    - NORD and Global Genes are tracking
  + Abigail Miller raised concern that it feels like we are using our voice to forward the few systems that using this system. This statement seems like we are only looking out for the systems that provide these systems. Are we doing it for certain businesses or that state of MN? Amy Gaviglio, Erica Barnes responded it is on behalf of the patients which is in within the councils purview.

# Legislative Update

Speaker: Amy Gaviglio

* The Council will be moved to the Minnesota Council of Disabilities
* We have revised our statute and added some clarification language of our duties
  + Policy Advocacy
  + Provide and Apply for grants
* In the Senate and House omnibus bill
* It is also a stand-alone in the Senate
* This would go into effect July 1st

# Adjournment

No Old Business

No New Business

Tom Blissenbach made the motion to adjurn, seconded. Meeting Adjourned at 4:30.

# Next steps: