

Rare Disease Council Meeting
Tuesday, March 29, 2022
10:00 AM – 12:00 PM

GoToWebinar Virtual Meeting

<https://attendee.gotowebinar.com/register/4184870319152057355>

Note: Council Members and presenters have been pre-registered and should not register again.

Meeting Minutes

Council Members Present (*ex officio members italicized*): Dr. Lisa Kaplowitz, Jana Monaco, Ijeoma Azubuko, Dr. Maureen Dempsey, Rebecca Goldbach, Dr. Stephen Green, Gregory Josephs, Dr. Peter Kasson, Holly Kearl, Dr. Tiffany Kimbrough, Susan Klees, Shannon McNeil, Dr. Richard Nicholas, Sarah Paciulli, Dr. Stephen Rich, Dr. Samantha Vergano, *Dr. Samantha Hollins, Jennifer Macdonald, Dr. John Morgan*

Council Members Absent: Megan O'Boyle

Virginia Department of Health (VDH) Staff Present: Christen Crews, Katherine Crawford

Dr. Lisa Kaplowitz, Chair, welcomed Council Members, and VDH staff performed the roll call. The Chair reviewed the Agenda and introduced Gregory Josephs, Council Member.

Mr. Josephs presented to the Council his personal experience as an individual living with a rare disease, and the importance of positive thinking, humor, and never giving up hope to his journey. He shared challenges and frustrations with receiving misdiagnoses from providers, and delays with receiving a timely and accurate diagnosis. He mentioned that the Orphan Drug Act of 1983 completely changed the face of therapeutics, yet still only 5% of 7,000 rare diseases have treatments. Mr. Josephs shared two videos: one of Jimmy Valvano accepting the Arthur Ashe award and a TED Talk by Lynn Hopkins titled "Curing Rare Disease is Possible." Mr. Josephs noted that diseases that cause the most harm do not always get the most research dollars, but to never give up. Mr. Josephs stated he is here to give hope to the rare disease community in Virginia.

Jana Monaco, Vice-Chair, introduced Delegate Kathleen Murphy, who sponsored legislation to create the Rare Disease Council in Virginia. Delegate Murphy mentioned how parents and caregivers of children with rare diseases had shared with her their struggles, such as issues with getting insurance coverage. She also listened to individuals from the Rare Disease Caucus share their experiences, and stated that the Rare Disease Council can help provide input and information to help guide the General Assembly to make meaningful change.

One individual, Carol Kelly, registered for public comment, but did not respond when invited to speak. No other individuals provided public comment.

The Chair and Vice-Chair then led Council Members in an Open Discussion period. Topics initially focused on developing plans for strategic planning and surveys. The Council requested that a subgroup meet before the next Council meeting to develop a draft mission, vision, and goals. These draft items would then be presented for full Council discussion and voting in the May 17 meeting. In addition to the Chair and Vice-Chair, three other Council members expressed interest in participating in the subgroup: Ijeoma Azubuko, Susan Klees, and Rebecca Goldbach. The Council requested that VDH staff assist with scheduling the subgroup meeting and with the strategic planning and survey development process.

The Vice-Chair introduced Marie Conley, Chair of the Pennsylvania Rare Disease Advisory Council (RDAC). Ms. Conley shared presentation slides, which included the RDAC's mission statement, vision statement, and an overview of how the council started and continues to evolve. She indicated that each state does things a little differently in terms of how a RDAC operates, though they all have a common denominator of advocacy for patients and families affected by rare diseases. She emphasized the importance of identifying what the RDAC could and could not do in order to help focus the council's efforts. Ms. Conley also gave a brief overview of how the RDAC created a patient needs assessment survey to gather data about the needs of families affected by rare diseases in Pennsylvania. Ms. Conley emphasized that this process will take time and shared that the RDAC worked with a PR firm. A current goal is to review initial survey results in a stakeholder summit in the fall to guide the RDAC's efforts.

The Vice-Chair introduced Annissa Reed, MPH, State Policy Manager, Eastern Region, National Organization for Rare Disorders (NORD). Ms. Reed introduced the NORD Annual State Report Card, which is a tool that the Council can use to help identify potential needs of individuals affected by rare diseases in the Commonwealth. After reviewing the rubric, categories, and ratings of the State Report Card, Ms. Reed indicated that the Council might want to focus on telehealth as a potential area needing improvement. She indicated that NORD re-evaluates states on an annual basis.

The Chair and Vice-Chair then led Council Members in an Open Discussion period. Topics included processes to develop a survey, a logo, and a website for the Council, as well as learning more about funding for the Council. Council members suggested learning more about the NORD Annual State Report Card to help inform discussion in the next meeting. The Council was also reminded that select member seats are currently accepting applications on the Secretary of the Commonwealth's website: the vacant hospital administrator seat, as well as members with terms expiring on 6/30/2022. The Chair adjourned the meeting after conclusion of the Open Discussion period.