Somewhere to Go for Adults with Childhood-Onset Rare Diseases: A Conversation About How We Can Fill Gaps in Care

October 19-20, 2023

Crystal City/Arlington, Virgina

Summary of Findings from a Two-Day Strategy Conference

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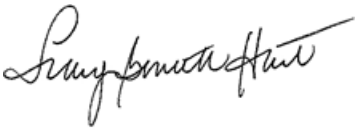
A Note from Tracy Hart, CEO, Osteogenesis Imperfecta Foundation:

Thank you so much to all of those who participated in the recent "Somewhere to Go for Adults with Childhood-Onset Rare Diseases: A Conversation About How We Can Fill Gaps in Care" meeting. Your valuable input is enabling us to prioritize the most important issues around transition of care and access to care for people with rare diseases so that we can make meaningful progress in addressing those issues. We will definitely be calling on you again…thank you for your support!

All my best,



Tracy Hart
Chief Executive Officer
Osteogenesis Imperfecta Foundation

Project Team

- **Tracy Hart**, Chief Executive Officer, Osteogenesis Imperfecta Foundation
- **Laura Tosi**, MD, Director, Bone Health Program at Children's National Medical Center
- **Eleanor Perfetto**, PhD, Professor, University of Maryland School of Pharmacy
- **Erika Carter**, Chief Program Officer, Osteogenesis Imperfecta Foundation
- **Melissa Bonardi**, Chief Development Officer, Osteogenesis Imperfecta Foundation; Director, Rare Bone Disease Alliance

Please cite this document as:

Hart T, Perfetto E, Bonardi M, Carter E, Connors S, Tosi L. (2024) Somewhere to Go for Adults with Childhood-Onset Rare Diseases: A Conversation About How We Can Fill Gaps in Care. Meeting Summary, October 19-20, 2023. Osteogenesis Imperfecta Foundation. http://www.oif.org/STG2024.

Overview

According to the NIH, ~30 million Americans live with a rare disease, which the Orphan Drug Act of 1963 defines as occurring when one person is diagnosed with an illness per every 200,000 people in a population. For the majority of people with a rare disease, the illness began in childhood. Although they may have received specialized pediatric care as children, upon adulthood, the healthcare system provides fewer resources for most. Millions of adults with childhood rare diseases face difficulty finding knowledgeable primary care and suitable specialty support. Moreover, the rare disease population is expanding rapidly. In 2016, the NIH identified 7,000 rare diseases: recent estimates place that number closer to 10,000. More than 90% of these disorders have no FDA-approved treatment. But improved care, and in some cases novel therapies, mean that many of these patients are living longer and reaching adulthood. This also means the dearth of appropriate adult-focused rare-disease care is a growing national problem.

The conference, 'Somewhere to Go for Adults with Childhood-Onset Rare Diseases: A Conversation About How We Can Fill Gaps in Care,' was held by the Osteogenesis Imperfecta (OI) Foundation over a two-day period (October 19-20, 2023) in Arlington, Virgina. The objectives behind this patient-centered conference were to:

1. Bring together a multistakeholder, multidisciplinary interested group for a working meeting on the topic of adult patients with rare, childhood-onset diseases who find themselves with "nowhere to go" for care.
2. Conduct a working meeting about adult persons with childhood-onset rare-diseases transitioning from pediatric to adult care to enumerate and describe the current gaps, barriers, and challenges they face, and the potential causes behind them.
3. Think strategically about solutions to address these gaps, barriers, and challenges, and
4. Prioritize which issues and/or solutions could be addressed collaboratively over the next 2-3 years by the convened group assembled (and others to be invited).

Through collaboration among the patients, caregivers, clinicians, and health researchers in a wide variety of fields, we aimed to identify common themes that transcend the specifics of individual rare diseases, recognizing it is not feasible or practical to have separate strategies for each disease. These foundational elements are essential to the longer-term tasks of devising solutions, establishing the metrics necessary to evaluate their actual performance, and understanding the conditions and incentives that facilitate adoption of adult care models that perform well for adult patients with childhood-onset rare diseases.

This document contains a summary of this two-day, working meeting, and presents the findings, work products, and recommendations that resulted.

The Two-day Working Meeting

Day One

Day one of the meeting consisted of opening remarks and four working sessions. The day opened with a welcome and introductions from members of the project team, including Tracy Hart, CEO of the OI Foundation; Laura Tosi, MD, Director of the Bone Health Program at Children's National; and Eleanor Perfetto, PhD, Professor at the University of Maryland.

Kara Ayers, PhD, provided a keynote from the 'voice of the community.' Kara described the agenda for the conference, including identifying gaps in care, reflecting on the potential harms and inequities perpetuated by the transition cliff, and discussed ways to intervene in systems and improve outcomes for adults with childhood-onset rare diseases. She spoke about level setting with our language, attitudes, ableism, and the disability paradox. A second keynote was provided by Annie Kennedy of the EveryLife Foundation for Rare Disease. Annie informed the group about the importance of supporting a lifetime of happiness, not just a happy childhood. She also spoke about her firsthand experiences in the rare-disease space and how she first came to understand what transition barriers were. These keynotes set the stage and motivated participants to get ready to roll up their sleeves.

The group then discussed and came to consensus on a goal statement for the meeting:

> "People with rare diseases deserve to have a smooth pathway when transitioning from pediatric care to adult care and through other, related transitions. Our goal is to find ways we, collectively, can make this happen."

Four working sessions followed. Each was moderated by Dr. Laura Tosi and began with speakers who introduced a care transition topic by providing firsthand experiences, describing challenges and barriers, discussing possible solutions, and offering examples of practices and models that might be scaled, replicated, and/or disseminated. Following each session's speakers, an open discussion session took place among the full group on the session topic, during which the group was asked to answer the following questions:

- What are the top 3 priorities that must be addressed in the next 2-3 years?
- What are 3 specific things we can do *collectively* now to achieve this vision?
- What role should patient organizations play?
- How can we best meet the needs of underserved populations?
- What does a coalition look like that will work collaboratively now to design and move a plan forward in the next 2-3 years?

Session 1: Envisioning the 21st Century: Centers of Excellence for People with Rare Disease – What Does That Look Like and How Do We Get There?

TjaMeika Davenport, Parent Navigator at Goldberg Center of Community Pediatric Health at Children's National, opened the first session by discussing what it is like to be a parent of a child with a rare disease and the difficulties faced being a caretaker for someone who has 'no place to go' when transitioning from pediatric to adult care.

Deborah Fowler, Founder and President of SoftBones, spoke on her organization's research into Centers of Excellence and whether it was a model they could endorse for hypophosphatasia (HPP). They concluded the model could work under the right guidelines, including working together to underscore the need for capacity

building of rare bone experts and make a viable business case for existing clinics as well as new doctors to see value in treating these patients.

Mena Scavina, DO, of Nemours, attended on behalf of Parent Project Muscular Dystrophy to discuss their certification process for centers throughout the U.S. She also discussed the addition of Transition to Adult Care in the Duchenne Muscular Dystrophy Diagnosis and Management, Part 1 guidelines, published in The Lancet, 2018. She spoke about the "ideal" or successful adult clinic, which would include access to state-of-the-art medical therapy, clinical trials, and technology, increased use of telehealth, transportation, caregiver support, academic and career counseling/opportunities, and financial and legal assistance, to name a few.

Kim Chapman, MD, PhD, wrapped up the presentations by reporting on the rare-disease transition model at Children's National, where she is the Director of the Mitochondrial Disorders Clinic.

A fireside chat was held during the midday break, led by Kara Ayers, PhD with guest speaker Lisa Iezzoni, MD, Professor of Medicine at Harvard Medical School. They discussed Dr. Iezzoni's research, which sheds light on how physicians view quality of life with a disability.

Session 2: Evidence for Decision Making: Leveraging Patient Data and the Patient Experience

Christopher "Buddy" Cassidy, MA, PhD Candidate in English Literature at UC Irvine, provided insight into natural history studies in trial design and regulatory approval.

Eileen King, PhD, Professor, UC Department of Pediatrics at Cincinnati Children's, discussed the patient role in ensuring reliable and relevant data for use in research that drives patient care. She emphasized the presence of longitudinal natural history studies, clinical trials, and quality improvement projects in rare disease research and provided insight on data sources based on patient care, data sources based on research, and improving the quality of clinical research data.

Eleanor Perfetto, PhD, Professor of Practice, Sciences, and Health Outcomes Research at the University of Maryland reported on patient-focused medical product development, which involves a systematic approach to ensure that patients' experience, perspectives, needs, and priorities are captured and meaningfully incorporated into the development and evaluation of medical products throughout the medical product life cycle.

Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement at the EveryLife Foundation for Rare Diseases, presented on the power and potential of data collaborations, emphasizing the need for institutions and organizations within the rare disease community to find the overlaps and opportunities to bring all the data sources together.

Session 3: Innovation in Care Coordination for People with Rare Diseases and Complex Needs

Kyle Mulroy, Founder of Washington Strategic Consulting, opened the third session of the day by speaking about the importance of care coordination and the patient perspective, and giving the audience a glimpse into his life with osteogenesis imperfecta.

Jenna Morgenstern-Gaines, CEO of Pocket RN, presented on bridging the gaps between home and health care with virtual nursing, or telehealth. Telehealth not only increases access for those in rural areas, but also for patients with rare diseases by helping them to quickly access specialists that could be hundreds of miles away.

David Ervin, CEO of Makom, discussed Medicaid managed care for individuals with intellectual and developmental disabilities (IDD). There are 7.5 million people in the United States with IDDs, 60% of which rely

on Medicaid for health insurance and access to long term services. The aim of Medicaid managed care is to reduce program costs and provide better utilization of health services; this includes transition incrementally.

Sandy Sandhaus, MD, PhD, Professor, Department of Medicine, Division of Pulmonary, Critical Care & Sleep Medicine, National Jewish Health, is also the Senior Medical Director of AlphaNet. He spoke about the AlphaNet model for patient engagement and coordination, which highlights patient serving patients and patient inclusion. This peer-to-peer model leads to higher adherence rates and program success at zero cost to the patient.

Session 4: Coalitions, Collaboratives and Working Together

Courtney Felle, Young Adult Rare Representatives (YARR) Program Manager, EveryLife Foundation for Rare Diseases, opened the fourth session by presenting on the YARR program, which has a goal of ensuring that young adults have a growing, diverse impact on public policy in the rare space. The program offers skill-building opportunities to foster growth in each individual's advocacy journey.

Debra Regier, MD, Division Chief, Genetics and Metabolism, Program Director, Medical Genetics Residency Program at Children's National spoke about the rare disease workforce and how transition is more than just medical.

Julie Hocker, Director, Public Health, Guidehouse (and Former US Commissioner on Disabilities), spoke about creating partnerships for effective collaboration and gave insight into leads on issues Federal agencies should respond to and how they are looking to leverage groups and partnerships like the ones forming within the conference itself.

Sara Struwe, CEO of the Spina Bifida Association, and Diana Gray, President and CEO of the Hydrocephalus Association, closed out session four by talking about the National Partnership for Pediatric to Adult Care Transition and the importance of coalition work.

The discussions and brainstorming from each working session were collated and main themes were identified and summarized. Three main theme areas emerged:

1. Issues: These consisted of topics that need to be addressed or overcome, including challenges and barriers, areas where information is lacking, and similar transition hurdles. (Table 1)
2. Possible actions: These consisted of general activities, possible solutions, and steps that could be taken to address the issues. (Table 2)
3. General principles: These consisted of a range of things to be mindful of when considering the issues and possible solutions, but that are not specific transition issues or solution per se. (Table 3)

Table 1. Main care transitions issues faced by adults with childhood-onset rare disease

- Reimbursement, insurance, code issues
- Lack of standards, guidelines, clinical practice guidelines, quality of care assessment
- Make transition a policy issue; a policy agenda is needed
- Lack of care coordination throughout transitioning
- Need to build and/or better leverage the workforce
- Leveraging AI and technology
- Leverage telemedicine
- Which provider is needed when? Who drives? Does not always have to be specialists. (Related to workforce issues)
- Lack of data standards and integration of electronic health records with other data sources
- Address patient preparedness/readiness for transition and possible challenges
- We don't know all of what's happening out there (who is doing what that can be joined, built on, leveraged, disseminated, scaled, etc.)

- Need to pilot/expand on existing, successful care models
- Need for more an appropriate diagnosis (for prevalence, evidence, cost estimates); are we looking at the tip of the iceberg?
- Addressing stigma as part of transitions
- Lack of mental health support during transitions
- Culture-of-care environment differs between pediatric and adult care
- Transition fatigue (so many different kinds of transitions at the same time)
- Different care transitions over time: pediatric to adult, adult to geriatric (Medicaid to Medicare)

Table 2. Possible Actions that Might be Taken to Smooth Care Transitions

- Form coalitions and collaborations
- Raise awareness
- Advocate
- Disseminate/educate
- Scale-up/replicate successes
- Establish new and leverage existing relationships
- Create an inventory/catalogue/SWAT analysis of what exists (assets, successes)
- Leverage existing expertise

Table 3. General Principles to be Considered in Care Transitions

- Consider equity: We don't have the capacity to solve all health equity issues, but we need to be mindful of underserved patients.
- Improved access is a core goal
- Leverage existing coalitions and collaborations
- Always ask: Which stakeholders are missing from the table?
- The patient voice, especially young voices, must be prominent
- Include "lay" colleagues, the entire team
- Leverage existing work, models, and success stories instead of reinventing the wheel
- Align priorities and visions within and across efforts
- Create and leverage win-win partnerships
- Thinks outside the box – not just "medical" approaches, partnerships, and services
- Consider patient privacy and control issues

Day Two

Day two opened with a recap of day one presented by Laura Tosi, MD. Two presentations on current activities and opportunities to address transition barriers were provided by:

Jeffrey P. Brosco, MD, PhD, Director, Division of Services for Children with Special Health Needs, MCHB, Health Resources & Services Administration (HRSA). Dr. Brosco discussed opportunities for the Maternal Child Health Bureau to support transition to adult systems of care.

Peggy McManus, MHS, President of The National Alliance to Advance Adolescent Health/Got Transition. Ms. McManus spoke on national recommendations for strengthening the adult primary care workforce for young adults with medical complexity.

Priorities, Takeaways, and Next Steps

Following the speaker presentations, one final group discussion session was held to review, revise, refine, and prioritize action steps for the future. The group refined themes from Tables 1-3 to create a full list of issues to be addressed in the next 2-3 years; 21 issues emerged from the discussion.(Table 4) Lastly, participants were asked to prioritize the main issues to be addressed, specifically thinking about what this group should take on, collectively, in the next 2-3 years to achieve the goal of a smooth pathway from childhood to adult-care transition. Each of the 26 remaining participants were asked to vote on his/her top three priorities on the list.

The top issues, prioritized by vote, are indicated in Table 4. Upon reflecting on the prioritization, the group discussed that work described in Topic 3, developing an inventory or catalogue on all currently happening, would be the most appropriate place to start given the information is needed for successful completion of almost all items on the list. The consensus was that this group should take the necessary next steps to become organized around an effort to create this resource, which would include establishing a collaborative team and leadership, creating a workplan, and seeking funding for support.

Table 4. Top Priority Action Steps to be Address in 2024-2026 to Smooth Care Transitions

Topic		Votes
1.	Development of transition success measures	12
2.	Better leverage the use of telemedicine to improve transitions	11
3.	***Gain better understanding of all that is happening in transition care. Develop an inventory/catalogue (who is doing what and where, that can be joined, built on, leveraged, disseminated, scaled)***	9
4.	Build and better leverage the workforce needed for the future to support transition care	8
5.	Improve patient (and family/caregiver) preparedness/readiness; develop an accessible toolbox that helps provide a soft landing for transitions	8
6.	Advance transitions as a policy issue; develop a policy agenda	7
7.	Data, data, data: Develop standards and advance integration of EHRs with other sources to strengthen evidence generation and dissemination of information	5
8.	Develop a research agenda on transitions	5
9.	Address reimbursement, insurance, coding issues	4
10.	Improve the lack of standards, guidance, clinical practice guidelines, quality of care measurement	3
11.	Improve mental health support during transitions	2
12.	Improve lack of care coordination throughout and after transitioning	1
13.	Leverage AI and other technologies to support transitions	1
14.	Address transition fatigue (so many kinds of transitions at once)	1
15.	Better support caregivers/family members/friends/care team/advocates throughout transitions	1
16.	Better inform decisions about which provider(s) are needed when. Who drives decisions? When to use specialists?	0
17	Pilot, expand on novel care models	0

18.	Increase and improve appropriate diagnosis (for prevalence, evidence, cost estimates). Might we be looking at the tip of the iceberg?	0
19	Consider culture of care differences between adult and pediatric care on transitions	0
20.	Consider stigma as part of transitions	0
21.	Consider the many different transitions happening over time: pediatric to adult to geriatric; Medicare to Medicaid	0

Tracy Hart and Laura Tosi closed the meeting by thanking the speakers and attendees for their participation, underscoring the importance of the recommendations, and initiating a discussion on next steps. A summary of the proceedings will be created and disseminated, both to participants and online, which includes the creation of this summary document and holding virtual follow-up meetings in 2024. Important work in 2024 will include taking initial steps to conduct the landscape analysis (gain a better understanding of all that is happening in transition care) and set in motion a process to create the inventory entitled “Care Transition Resource Repository.” Additional activities will include expanding our network of partners, ensuring that we are avoiding duplication of existing work, and developing strategies for the future to address the additional items on the recommendation list.

Appendix

Speakers:

- **Kara Ayers**, PhD, Associate Director, University of Cincinnati, Center for Excellence in Developmental Disabilities
- **Jeffrey P. Brosco**, MD, PhD, Director, Division of Services for Children with Special Health Needs, MCHB, Health Resources & Service Administration (HRSA)
- **Christopher "Buddy" Cassidy**, MA, PhD Candidate in English Literature, UC Irvine
- **Kim Chapman**, MD, PhD, Director of the Mitochondrial Disorders Clinic, Children's National
- **TjaMeika Davenport**, Parent Navigator at Goldberg Center for Community Pediatric Health, Children's National
- **David Ervin**, CEO, Makom
- **Courtney Felle**, Young Adult Rare Representatives (YARR) Program Manager, EveryLife Foundation for Rare Diseases
- **Deborah Fowler**, Founder and President, SoftBones
- **Diana Gray**, President and CEO, Hydrocephalus Association
- **Tracy Hart**, CEO, Osteogenesis Imperfecta Foundation
- **Julie Hocker**, Director, Public Health, Guidehouse and Former US Commissioner on Disabilities
- **Lisa Iezzoni**, MD, Professor of Medicine, Harvard Medical School
- **Annie Kennedy**, Chief of Policy, Advocacy, and Patient Engagement, EveryLife Foundation for Rare Diseases
- **Eileen King**, PhD, Professor, UC Department of Pediatrics, Cincinnati Children's
- **Peggy McManus**, MHS, President of the National Alliance to Advance Adolescent Health/Got Transition
- **Jenna Morgenstern-Gaines**, CEO, Pocket RN
- **Kyle Mulroy**, Founder, Washington Strategic Consulting
- **Eleanor Perfetto**, PhD, Professor of Practice, Sciences, and Health Outcomes Research, University of Maryland
- **Debra Regier**, MD, Division Chief, Genetics and Metabolism, Program Director, Medical Genetics Residency Program, Children's National
- **Robert "Sandy" Sandhaus**, MD, PhD, Professor, Department of Medicine, Division of Pulmonary, Critical Care & Sleep Medicine, National Jewish Health
- **Mena Scavina**, DO, Nemours, PPMD
- **Sara Struwe**, CEO, Spina Bifida Association
- **Laura Tosi**, MD (Chair), Director, Bone Health Program, Children's National

Sponsors:

- Foundation to Eradicate Duchenne
- Guidehouse
- Orthopaedic Research Society (ORS)

Attendees:

First Name	Last Name	Organization
Kara	Ayers	Cincinnati Children's
Jeff	Brosco	HRSA
Susan	Bukata	Orthopedic Research Society
Hannah	Busey	Guidehouse
Gwen	Carmon	March of Dimes
Christopher "Buddy"	Cassidy	UC Irvine
Kim	Chapman	Children's National
Kathryn	Cowie	YARR and Nested Knowledge
TjaMeika	Davenport	Children's National
Mark	Delvaux	AlphaNet, Inc
David	Ervin	Makom
Courtney	Felle	EveryLife Foundation for Rare Diseases
Doug	Fessler	ASBMR
Debbie	Forrest	Venable
Deborah	Fowler	Soft bones
Eric	Gascho	National Health Council
Claire	Gill	Bone Health and Osteoporosis Foundation
Denise	Goodbar	Soft Bones
Diana	Gray	Hydrocephalus Association
Andrea	Gropman	Children's National
Lauren	Hahn	Children's National
Nancy	Harry	Children's National
Tracy	Hart	Osteogenesis Imperfecta Foundation
Julie	Hocker	Guidehouse
Annie	Kennedy	Everylife Foundation
Eileen	King	Cincinnati Children's
Tracy	King	NICHD - NIH
Ellyn	Kodroff	CURED Foundation
Adrienne	McBride	FDMAS Alliance
Peggy	McManus	The National Alliance to Advance Adolescent Health
Jenna	Morgenstern-Gaines	Pocket RN
Kyle	Mulroy	Washington Strategic Consulting
Andrew	Pennington	Children's National
Eleanor	Perfetto	University of Maryland
Debra	Regier	Children's National
Kendall	Reid	Children's National
Allie	Roverud	Makom
Sandy	Sandhaus	National Jewish Health
Mena	Scavina	Nemours
Kathryn	Smith	HRC
Sarah	Struwe	Spina Bifida Association

Reid	Sutton	Texas Children's
Judy	Thibadeau	Spina Bifida Association
Laura	Tosi	Children's National
Taylor	Turner	The Federal Group, Inc.
Kristen	Wheeden	United Porphyrias Association
Dana	Wood	N/A
Joel	Wood	N/A

Agenda:

OSTEOGENESIS IMPERFECTA FOUNDATION
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Somewhere to Go for Adults with Childhood-Onset Rare Diseases:
A Conversation About How We Can Fill Gaps in Care
October 19-20, 2023
Crystal City/Arlington, VA

AGENDA

Thursday, October 19

9:00AM – 9:15AM **Welcome and Introductions:**
Tracy Hart, CEO, Osteogenesis Imperfecta Foundation
Laura Tosi, MD, Director, Bone Health Program, Children's National
Eleanor Perfetto, PhD, Professor of Practice, Sciences, and Health Outcomes Research, University of Maryland

9:15AM – 9:30AM **Voice of the Community**
Kara Ayers, PhD, Associate Director, University of Cincinnati, Center for Excellence in Developmental Disabilities

9:30AM – 10:00AM **Keynote Speaker:**
***Back to the Future*: Supporting a Lifetime of Happiness, not Just a Happy Childhood**
Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement, EveryLife Foundation for Rare Diseases

SESSION 1: **Envisioning the 21st Century: Centers of Excellence for People with Rare Disease – What Does That Look Like and How Do We Get There?**

10:00AM – 10:15AM **Experiencing No Place to Go**
TjaMeika Davenport, Parent Navigator at Goldberg Center for Community Pediatric Health, Children's National

10:15AM – 10:30AM **Centers of Excellence – Will They Work for Our Community?**
Deborah Fowler, Founder and President, SoftBones

10:30AM – 10:45AM **Center Certification: Parent Project Muscular Dystrophy**
Mena Scavina, DO, Nemours, PPMD

10:45AM – 11:00AM **Rare Disease – Transition Model**
Kim Chapman, MD, PhD, Director of the Mitochondrial Disorders Clinic, Children's National

11:00AM – 11:30AM **Session 1: Group Discussion**

11:30AM – 12:45PM **Lunch**

12:00PM – 12:45PM **I Am Not the Doctor for You**
Lisa Iezzoni, MD, Professor of Medicine, Harvard Medical School
Kara Ayers, PhD, Associate Director, University of Cincinnati, Center for Excellence in Developmental Disabilities

SESSION 2:	**Evidence for Decision Making: Leveraging Patient Data and the Patient Experience**
12:45PM – 1:00PM	**It's Only Natural Getting the Most Out of Natural History Studies in Trial Design and Regulatory Approval** Christopher "Buddy" Cassidy, MA, PhD Candidate in English Literature, UC Irvine
1:00PM – 1:15PM	**Patient Role in Ensuring Reliable and Relevant Data for Use in Research that Drives Patient Care** Eileen King, PhD, Professor, UC Department of Pediatrics, Cincinnati Children's
1:15PM – 1:30PM	**Patient Focused Medical Product Development** Eleanor Perfetto, PhD, Professor of Practice, Sciences, and Health Outcomes Research, University of Maryland
1:30PM – 1:45PM	**The Power & Potential of Data Collaborations** Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement, EveryLife Foundation for Rare Diseases
1:45PM – 2:15PM	**Session 2: Group Discussion**
2:15PM – 2:30PM	**Break**
SESSION 3:	**Innovation in Care Coordination for People with Rare Diseases and Complex Needs**
2:30PM – 2:45PM	**The Importance of Care Coordination – A Patient's Perspective** Kyle Mulroy, Founder, Washington Strategic Consulting
2:45PM – 3:00PM	**Bridging the Gap with Virtual Nursing** Jenna Morgenstern-Gaines, CEO, Pocket RN
3:00PM – 3:15PM	**Managed Care for Adults with IDD: Panacea or Predicament?** David Ervin, CEO, Makom
3:15PM – 3:30PM	**The AlphaNet Model for Patient Engagement** Sandy Sandhaus, MD, PhD, Professor, Department of Medicine, Division of Pulmonary, Critical Care & Sleep Medicine, National Jewish Health
3:30PM – 4:00PM	**Session 3: Group Discussion**
SESSION 4:	**Coalitions, Collaboratives and Working Together**
4:00PM – 4:15PM	**Stronger Together** Courtney Felle, Young Adult Rare Representatives (YARR) Program Manager, EveryLife Foundation for Rare Diseases
4:15PM – 4:30PM	**The Right Healthcare Workforce for Adult Complex Care** Debra Regier, MD, Division Chief, Genetics and Metabolism Program Director, Medical Genetics Residency Program, Children's National
4:30PM – 4:45PM	**Creating Partnerships for Effective Collaborations** Julie Hocker, Director, Public Health, Guidehouse and Former US Commissioner on Disabilities
4:45PM – 5:00PM	**NPPACT and the Importance of Coalition Work** Sara Struwe, CEO, Spina Bifida Association Diana Gray, President and CEO, Hydrocephalus Association
5:00PM – 5:30PM	**Session 4: Group Discussion**
5:30PM – 7:00PM	**Break**

7:00PM – 9:00PM	**Dinner and Recap of Day:** Laura Tosi, MD, Director, Bone Health Program, Children's National Eleanor Perfetto, PhD, Professor of Practice, Sciences, and Health Outcomes Research, University of Maryland

Friday, October 20

8:00AM – 8:30AM	**Breakfast**
8:30AM – 8:40AM	**Welcome and Recap of Day 1:** Laura Tosi, MD, Director, Bone Health Program, Children's National
8:40AM – 9:05AM	**What Can the Maternal Child Health Bureau (MCHB) Do to Support Transition to Adult Systems of Care?** Jeffrey P. Brosco, MD, PhD, Director, Division of Services for Children with Special Health Needs, MCHB, Health Resources & Services Administration (HRSA)
9:05AM – 9:30AM	**National Recommendations for Strengthening the Adult Primary Care Workforce for Young Adults with Medical Complexity** Margaret McManus, MHS, President of The National Alliance to Advance Adolescent Health
9:30AM – 10:15AM	**Where We Landed: Review and Open Discussion:** Eleanor Perfetto, PhD, Professor of Practice, Sciences, and Health Outcomes Research, University of Maryland
10:15AM – 11:30AM	**Priorities from Meeting and Group Working Session**
11:30AM – 11:45AM	**Next Steps: Follow Up and Follow Through:** Laura Tosi, MD, Director, Bone Health Program, Children's National

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