




Epilepsy Community at an Inflection Point: Translating Research Toward Curing the Epilepsies and Improving Patient Outcomes

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**Ilene Penn Miller, JD, LLM^{1*}, JayEtta Hecker, MS²,
Brandy Fureman, PhD³, Mary Anne Meskis⁴, Steve Roberds, PhD⁵ ,
Monika Jones, JD⁶, Heidi Grabenstatter, PhD⁷, Vanessa Vogel-
Farley, BAA⁸, and Laura Lubbers, PhD⁹**

¹ Rare Epilepsy Network, Bethesda, MD, USA

² Wishes for Elliott, Washington, DC, USA

³ Epilepsy Foundation, Landover, MD, USA

⁴ Dravet Syndrome Foundation, Cherry Hill, NJ, USA

⁵ TSC Alliance, Silver Spring, MD, USA

⁶ The Brain Recovery Project Childhood Epilepsy Surgery Foundation, Los Angeles, CA, USA

⁷ International Foundation for CDKL5 Research, Wadsworth, OH, USA

⁸ Dup15q Alliance, Highland Park, IL, USA

⁹ CURE Epilepsy, Chicago, IL, USA

*Correspondence: Ilene Miller, JD, LLM, Rare Epilepsy Network, 6716 Vendome Terr, Bethesda, MD 20817, USA.

Email: ilenepennmiller@gmail.com

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The 2021 Curing the Epilepsies Conference brought together patient advocacy organizations, professional societies, researchers, and clinicians to discuss transformative priorities that could significantly advance research toward cures and improved health outcomes for people with epilepsy. During the three-day conference, key gaps and opportunities were identified and have been summarized in separate editorials from the Epilepsy Research Benchmark Stewards and patient advocacy organizations. Patient advocates identified the following priorities as critical to advancing research on epilepsy, (1) integrating epilepsy care and research; (2) reducing health disparities for underserved communities; (3) improving measurement and tracking of patient outcomes; and (4) establishing national data on burden, incidence, prevalence, and mortality. Key to driving these transformative priorities is the development of a National Plan. This strategic plan would align and integrate basic, translational, clinical, population, and implementation research with the infrastructure, incentives, and resources to put the nation on a path to developing cures and improving the quality of life for every person touched by epilepsy.

Over two decades ago, a movement led by advocacy organizations, professional societies, and governmental agencies challenged the epilepsy research community to focus on the

difficult problem of curing epilepsy, defined as “no seizures, no side effects, and prevention for those at risk.” This challenge to take the science beyond controlling seizures, the central



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symptom of epilepsy, to actually curing and preventing epilepsy resulted in Curing the Epilepsies (CTE) conferences and a series of Epilepsy Research Benchmarks that have guided research strategies in the field.

The research investments over these two decades have resulted in enormous progress in understanding the causes of the epilepsies (particularly genetic and immune-related causes) and in the basic mechanisms of epileptogenesis. Notable gains have also been made in understanding comorbidities associated with the epilepsies, the increased risks of early mortality (from SUDEP, suicide, and other causes), in technological improvements in epilepsy diagnosis (especially in neuroimaging and genetic testing), and anti-seizure treatment (especially in surgery, dietary therapy, and devices). Discovery and research progress is often incremental and takes significant time and investment which is understood by advocates and researchers alike.

Leveraging the exciting progress over the last 20+ years, as reflected in the revised Benchmarks, has brought us to another inflection point in the field. Our challenge today is to work collaboratively to create the plan, the infrastructure, and the expanded partnerships needed to rapidly translate these paradigm-shifting scientific discoveries into better health outcomes for adults, children, and families living with epilepsy.

An Inflection Point for CTE: Translating Research Gains Into Improved Patient Outcomes

“There has been remarkable, but steady progress in epilepsy research...[but] there have been no significant changes to prognosis, treatment or outcomes for patients.”

-Eric Marsh, MD, PhD, Associate Professor of Neurology, Children’s Hospital of Philadelphia and Clinical Director, Orphan Disease Center

The transformative steps needed to translate remarkable scientific progress into improved outcomes for all those living with the epilepsies were underscored in a keynote by one of the leaders of the process. Expanding the focus of the recent CTE conference to consider transformative opportunities resulted in fresh thinking and new strategies.

Many themes spanned the conference, including the need for innovative strategies to improve the culture of the epilepsy community by breaking down silos, incentivizing a culture of knowledge sharing, improved data-sharing, and engaging the next generation of scientists and researchers. Other scientific opportunities were explored including better understanding mechanisms, developing and validating biomarkers, improving preclinical models, and utilizing new tools from the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative.¹ All of these important opportunities were captured by the Stewards Committee Co-Chairs in their companion editorial, and we agree they represent new research priorities for moving forward.

As patient advocates, we believe we need a laser focus on a few overarching opportunities which together hold promise for improving the quality of life of all those living with epilepsy. Advocates—representing the lived experience of the epilepsies as patients and caregivers—spoke about the need for transformative change in the epilepsy community.

Key Advocate Quotes from 2021 CTE

“In no other disease space would removing half a child’s brain be an acceptable cure, but for some reason, we accept this for epilepsy.”

-Monika Jones, JD, Parent, Advocate, and Founder Executive Director, The Brain Recovery Project

“It’s not about moving from 2 seizures a week to 1 seizure a week. It’s about moving multiple seizures per day to a situation where we can live a life without fear; with having a conversation; with enjoying parks and so many other things that most of us take for granted.”

-Steve Roberds, PhD, Chief Scientific Officer, TSC Alliance

“I left my dream job as an equity analyst and raised over \$2m in 18 months... It’s unsustainable for families – we don’t have years – our urgency is like AIDS or COVID.”

-Amber Freed, CFA, Parent, Advocate and Co-Founder SLC6A1 Connect

“Do my children’s lives depend on a mouse? And do I, their mother, have to make it?”

-Kim Nye, Parent, Advocate, Founder and Executive Director of TESS Research Foundation

“Time is brain and we’ve lost too much of both. It’s time for Covid- level collaboration that includes a National Strategy to cure the epilepsies.”

-Gabrielle Conecker, MPH, Parent, Advocate, and Co-Founder Wishes for Elliott & DEE-P Connections

Focused transformative actions are required to reduce the persistently high share of patients unresponsive to treatment, the debilitating effects of epilepsy, and high rates of mortality, as well as to increase the availability of disease altering and preventative treatments. We call for prioritizing four overriding opportunities that emerged from the conference—two on improving care and two on improving data. All four areas were widely discussed during the conference and were recognized in the Stewards Committee Co-Chairs companion editorial.

Improving Care, Clinical Research, and Coverage

Integrating Epilepsy Care and Research

Integrating clinical care and research is needed to capture and share learnings from each patient. This priority incorporates the frequent refrain during the conference about the need for large multi-centered longitudinal studies and combines clinical research with epidemiological research with timely distillation of insights being captured and shared from each case. This model offers the

mechanism and structure for implementing other improvements including needed advancements in targeted treatments, accelerating identification and application of various biomarkers, integrating data to drive better clinical care and research, and facilitating phenotyping, biospecimen analysis, and applied and highly nimble data repositories.

Integrating care and research also provides an efficient and effective opportunity to provide new structures that value and reward a culture of collaboration and sharing, increasing the opportunities and incentives for more outcome-focused practices. Integrated care and research centers would facilitate the development, refinement, validation, and application of endpoints that are monitorable, measurable, and meaningful to patients and families. There are multiple existing and proven models for integrating care and research, as recognized by the Benchmark Stewards. The epilepsy community has the foundation of many ongoing collaborative efforts to build on when implementing an integrated care and research approach including the Pediatric Epilepsy Research Consortium (PERC), Epilepsy Learning Healthcare System (ELHS), Pediatric Epilepsy Learning Healthcare System (PELHS), National Association of Epilepsy Centers (NAEC), and other consortia and programs. A comprehensive plan can apply lessons learned, engage innovative leaders, and chart the path for building a new infrastructure combining cutting-edge clinical care and research.

Reducing Health Disparities for Underserved Communities

Addressing the needs of underserved populations experiencing disparities in healthcare treatment and outcomes requires a comprehensive and strategic approach. Communities most affected need to be engaged in developing the strategy. Further, more public health and implementation research—with input from the communities affected and researchers—is needed. Exemplary models with targeted efforts exist in both asthma and vaccination campaigns. Similarly, young scientists and new researchers from underserved populations need to be engaged. A root cause analysis is needed to understand these disparities and marshal a comprehensive national strategy to correct the unacceptably high disparities in health outcomes for minority, rural, rare, low income and other underserved populations.

Improving Data to Inform Care and Monitoring

Improving Measurement and Tracking of Patient Outcomes

Patient-centered outcome measures which capture overall quality of life need to be developed, refined, validated, and put into ongoing use. These measures are critical to better clinical

care, clinical trials, and research in evidentiary data on real-world patient experience. A national strategy is needed to forge the many disparate efforts to develop and improve outcome measures into a cohesive effort optimized for efficiency, coordination, and application.

Establishing National Data on Burden, Incidence, Prevalence, and Mortality

National collection of key epilepsy statistics on incidence, prevalence, and mortality by age, diagnosis, ethnicity, race, socioeconomic status, and more needs to be prioritized. While it is estimated that epilepsy will affect 1 in 26 Americans over their lifetime³, with lifelong and profound impacts on so many, the nation lacks detailed standardized, reliable, and comprehensive data on the population burden of epilepsy.

Opportunities to Accelerate Research and Patient-Centered Outcomes for the Epilepsy Community

The collective activities related to the review and revision of the Benchmarks and development and execution of the 2021 CTE conference brought researchers, clinicians, and advocates together as never before. This process has brought into focus many new opportunities to accelerate research and result in improved patient-centered outcomes for the epilepsy community. Patient and professional advocates and the Benchmark Stewards agree that there are immediate actions that can be taken to accelerate progress while increasing inclusivity, including

- *identifying immediate strategies* to enhance the patient voice in all aspects of research planning, using principles employed by other established government, private, and nonprofit funding organizations;
- *partnering across the epilepsy community to extend and deepen the conversations on specific research topics and areas* from the 2021 conference through workshops and annual discussion forums at society meetings; and
- *revisiting of the Benchmarks and CTE conferences more frequently* to drive transformative change and to adapt priorities to evolving times.

Implementing these opportunities would connect the epilepsy research community and forge new understanding to advance research. However, to truly accelerate progress and cure the epilepsies, the entire epilepsy community must come together to create a comprehensive strategy, the infrastructure, and the expanded partnerships to rapidly translate scientific discoveries into better health outcomes.

Thanks to the culmination of the hard work and support of the many stakeholders in the epilepsy community over the last 20 years—including the American Epilepsy Society (AES), National Institutes of Health, National Institute of Neurological



Disorders and Stroke, the NAEC, clinicians, researchers, industry, and patient organizations—our community is now positioned to begin developing an aggressive and coordinated National Plan to address epilepsy, including strategy and implementation that will accelerate progress and will allow us to provide better clinical care and services to those living with epilepsy and their families. Inspired in great part by the collaborative spirit of this year's meeting, the patient advocacy community proposes a call-to-action to bring together a stakeholder planning committee to prioritize the development of this comprehensive strategy.

A National Plan would provide an effective vehicle for addressing the many opportunities explored during the CTE 2021 process. This includes the areas we believe need to be prioritized including (1) integrating epilepsy care and research; (2) reducing health disparities for underserved communities; (3) improving measurement and tracking of patient outcomes; and (4) establishing national data on burden, incidence, prevalence, and mortality—among other priorities. A National Plan provides the opportunity to leverage principles and operating models outside of epilepsy research such as those used to advance the research and treatment of pediatric oncology and Alzheimer's disease. Research and outcomes for these diseases have each benefited from development of a comprehensive national plan and strategy and deepened federal involvement and investment to address the disease. This presents a major new area for exploration and learning highlighted by advocates, as well as the Benchmark Stewards.

“Our strategy for my sister's care was to wait and hope, and that is no strategy.”

Laura Lubbers, PhD, Advocate, Sibling and Chief Scientific Officer, CURE Epilepsy

The epilepsy research and advocacy communities have a strong history of working together to outline concerns faced by those affected by epilepsy through development of strategies, such as the Institute of Medicine report *Epilepsy Across the Spectrum: Promoting Health and Understanding* and the development of the Benchmarks themselves.² Learning from that experience, we recognize an effective National Plan to address epilepsy will require inclusion of a strategy, implementation steps, oversight, monitoring, and measuring progress against agreed upon milestones.

The 2021 CTE conference has catalyzed our shared responsibility to use our collective strengths—the lived experience of the advocates, the cutting-edge scientific discoveries of

the researchers, the practice insights of the clinicians, and the broad commitment of industry—to explore new and innovative approaches to transform the lives of those affected by the epilepsies. Working together, we can chart the course for aligning the infrastructure, incentives, and resources to put the nation on a path to improving the quality of life for every person touched by epilepsy. Epilepsy knows no geographic borders. A strong National Plan will also allow the opportunity to enhance research collaboration between countries and enable innovation worldwide—and finally “cure the epilepsies.”

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ORCID iD

Steve Roberds  <https://orcid.org/0000-0003-0676-272X>

References

1. *The brain research through advancing innovative neuro-technologies® (BRAIN) initiative*. <https://braininitiative.nih.gov>. Accessed May 1, 2021.
2. Institute of Medicine (US). Committee on the public health dimensions of the epilepsies. In: England MJ, Liverman CT, Schultz AM, Strawbridge LM, eds. *Epilepsy across the Spectrum: Promoting Health and Understanding*. Washington, DC: National Academies Press (US); 2012.
3. Hesdorffer, Logroscino, Benn, Katri, Cascino and Hauser. Estimating risk for developing epilepsy: A population-based study in Rochester Minnesota. *Neurology* 2011;76:23-27.