

RESEARCH CP

SETTING A PATIENT-CENTERED RESEARCH AGENDA FOR CEREBRAL PALSY

CPNOW
Advancing Neurorecovery

 **CPRN**
CEREBRAL PALSY RESEARCH NETWORK



Paul Gross
Chairman and Founder
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Cerebral Palsy Research Network Founding

- NIH Workshop on cerebral palsy in November 2014 Key Takeaways
 1. Start a national CP registry
 2. Pursue Comparative Effectiveness Research question
 3. Increase the study of adults
 4. Bring more young clinician scientists into the field
 5. Advance basic and translational science for CP
- CPRN founded to do 1-4
- Merger of two core experiences: HCRN and Learn From Every Patient
- Patient-centered from the outset

Research CP: Finding out what the patient community cares about

- *Research CP* was conceived of to help CPRN and the greater research community focus its research efforts.
- Parent advocates and adults with CP continue to struggle with decision making because of a lack of comparative research.
- Communicate CP research priorities to medical professionals, granting agencies, government agencies and the insurance industry.

Research CP Objectives – PCORI Application

1. Educate patients, caregivers and clinicians via webinars
2. Survey the patient and provider on research priorities for CP
3. Convene a workshop to set a research agenda
4. Publish a whitepaper outlining the community's shared comparative effectiveness and quality agenda for cerebral palsy.

DEVELOPMENTAL MEDICINE & CHILD NEUROLOGY ORIGINAL ARTICLE

Setting a patient-centered research agenda for cerebral palsy: a participatory action research initiative

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ABBREVIATION
CPRN Cerebral Palsy Research Network

AIM To establish a patient-centered research agenda for cerebral palsy (CP).
METHOD We engaged a large cross-section of the extended community of people living with CP and those providing healthcare to people with CP ('the community') in an educational series and collaborative survey platform to establish an initial list of prioritized research ideas. After online workshops, a facilitated Delphi process was used to select the 20 highest priorities. Select participants attended an in-person workshop to provide comment and work toward consensus of research priorities.
RESULTS A research agenda for CP was developed by the community, which included consumers, clinicians, and researchers interested in advancing the established research agenda. The results included the top 16 research concepts produced by the process to shape and steward the research agenda, and an engaged cross-section of the community.
INTERPRETATION It has been shown that proactively engaging consumers with clinical researchers may provide more meaningful research for the community. This study suggests that future research should have more focus on interventions and outcomes across the lifespan with increased emphasis on the following outcome measures: function, quality of life, and participation.

Cerebral palsy (CP) is an umbrella term for non-progressive, permanent brain disorders that affect development of movement and posture and precipitate a host of secondary and chronic comorbidities.¹ A recent article reports that public funding of CP research between 2001 and 2013 increased, but that funding for studies of clinical interventions comprised only 19 percent of the funding and CP in adulthood comprised only 4 percent.² A survey of an online community of parents and caregivers (n=1214) of children with CP conducted by the nonprofit organization CP NOW found that available medical information was judged by survey respondents (parents) to be inadequate to guide medical decision-making for children with CP.³ Perceived inadequacy of available information reported by survey respondents may be attributable to the diversity of clinical presentations in this population, the broad array of treatments and medical providers involved in care of persons with CP, the lack of comparative effectiveness research for treatments, and the long-term, progressive nature of CP sequelae. Regardless of cause, perceived inadequacy of information points to the need for a

research agenda involving the extended community of people living with CP and those providing healthcare to people with CP that informs medical decision-making for persons with CP.
Patient and caregiver involvement in the development of research is vital to producing research that improves the health and well-being of all patient populations,⁴ and, more specifically, for those with disabilities.⁵ However, efforts toward this end are somewhat limited. To date, three groups have published the results of their efforts that included persons with CP and their caregivers in the development of research agendas. Two of these efforts were focused broadly on neurodisability rather than specifically on CP and included caregivers and persons with several neurodevelopmental conditions.^{6,7} The third effort focused specifically on CP in Australia⁸ and may have limited generalizability outside of the Australian healthcare context. In addition, the McInyre et al.⁸ study was conducted 10 years ago and differed from others in that it did not include an in-person meeting. It is possible that these meetings may foster a deeper understanding of various perspectives⁹ since

Survey the patient and provider community

- Codigital to generate, prioritize and refine ideas

The screenshot displays the Codigital survey interface. On the left, a 'Rank Ideas' section shows two ideas for ranking: 'What is the prevalence of mental illness in patients diagnosed with Cerebral Palsy.' and 'What is the right type and amount during childhood to optimize skeletal health in adulthood for people of varying motor ability?'. Each idea has an 'Edit' button and a 'Vote this idea' button. An 'OR' button is positioned between the two ideas. On the right, a summary panel shows the survey objective: 'What research questions are most important to you?'. It includes statistics: 15 Contributors, 16 Ideas, 8 Edits, and 198 Votes. A pie chart shows the distribution of contributions, and a bar chart indicates the user's contribution is 3rd. The survey is set to last for 4 days.

Rank Ideas 7

Please click on the idea that deserves the higher rank

What research questions are most important to you?

What is the prevalence of mental illness in patients diagnosed with Cerebral Palsy. [Edit](#)

OR

What is the right type and amount during childhood to optimize skeletal health in adulthood for people of varying motor ability? [Edit](#)

[Vote this idea ✓](#) [Vote this idea](#)

OBJECTIVE

What research questions are most important to you? 4 days to go

Contributors **15** Your Rating **52.2** 3rd

[+ ADD](#) [RANK 7](#) [RESOLVE 3](#)

1 What interventions, including surgeries, injections, medications and therapies are associated with better functional outcomes controlling for child CP and health characteristics? [Gen 2](#) [Edits 0](#) [Edit](#)

2 What is the right type and amount of exercise during childhood to optimize skeletal health in adulthood for people of varying levels of gross motor ability? [Gen 1](#) [Edits 2](#) [Edit](#)

3 Are "intensive" therapies more effective than less intense but over a longer period of time? I.e. Would an intensive therapy protocol 5 days/week for 3 hours/day for 3 weeks be more effective than a standard 1 hour/day for 2 days/week for a year? [Gen 1](#) [Edits 0](#) [Edit](#)

4 Comparative effectiveness of SPML v. SEMLS for various cohorts of kids with 2, 5 and 10 year outcomes. [Edit](#)

Summary [Top Contributors](#) [Getting Started](#)

15 Contributors **16** Ideas **8** Edits **198** Votes

Your Contribution **3rd**

What do adults with CP want to know?



“My biggest challenge is understanding what is happening within my body. It appears over the last couple years that my muscle has deteriorated. I expected some deterioration, but not quite this early. I would like to determine if this is reversible or how much it will worsen.”

-Russell Thompson

Why patient-centered research?



“If our research aims to improve the lives of people with CP, it seems self-evident that we should understand in what way they want their lives improved. Partnering with people with CP and their carers, will inform the questions we ask, the methods and measures we use to answer them, and facilitate the means by which we can adopt or implement what will most benefit them.”

-Unni Narayanan, MD

Common observations among participants

- A desire to keep the momentum of the partnership moving forward.
- Longitudinal studies across the age span and the clinical spectrum of CP should drive research.
- The importance of the adult perspective including the cascading loss of function.
- Emphasizing participation as an important outcome measure.

Limitations

- Missing adolescent voice
- Codigital favored broad topics over narrower topics relevant to smaller slice of participants
- Selection bias common to social media targets
- Results influenced by relative participation of different groups although consumers (parent, person with CP, advocate) contributed >60%

Genetic Causes of Cerebral Palsy

- Funded by NINDS/NIH
 - Precision Medicine Initiative
- CPRN sites will refer patients with unknown etiology
- Combined power of genetic analysis and clinical description and follow up will open new vistas of understanding in CP—long term outcomes, treatment outcomes



Michael Krueer, MD
Phoenix Children's Hospital

Key Takeaways

- Create space and safety for patient / clinician researcher interaction
- Plan for iterative process to overcome overly broad research ideas
- Partner clinician researchers with a track record for funded research