



AT THE FOREFRONT

UChicago
Medicine



Improving Care for Central Nervous System Congenital Anomalies

Margaret Hackett Family Program Progress Report

October 2020



The University of Chicago Medicine provides outstanding care for people with central nervous system congenital anomalies. Thanks to your partnership, we have established the Margaret Hackett Family Program (MHFP) as a hub for patient care and research aimed at improving outcomes and quality of life for people impacted by these conditions. With your support, our faculty and staff are providing a warm and compassionate destination for care, coordinating a research consortium, and constructing a database of knowledge to support clinical and translational studies. We are pleased to present this update on recent progress that has been made possible through your partnership.

Pillar 1: Patient-Focused Outreach and Support

Over the past six months, our team has advanced several initiatives focused on supporting patients throughout their care.

Facilitating Awareness of the Margaret Hackett Family Program

Outreach to Third Party Organizations

We are working with third-party organizations to create a network that will improve access to resources for patients and families impacted by central nervous system (CNS) anomalies. The Margaret Hackett Family Program (MHFP) will be available to these organizations as a source of specialized information, care, and support. Over the past six months, two hydrocephalus organizations, the Hydrocephalus Association and Hydrocephalus Kids have added the MHFP to their directories of Chicago-area programs and centers specializing in hydrocephalus. Directories like these help patients and families locate and connect with programs related to specific conditions.

Website Content Development

The team has finished building the [MHFP website](#), which is now live. The site presents background about the program and provides educational content to help patients and their families learn about CNS anomalies. It also offers a simple online form for requesting an appointment.

The UChicago Medicine website also highlights the MHFP as a resource for those impacted by CNS anomalies. The [main page about CNS anomalies](#) features an overview of the program, contact information, and a link to the program's website. Information is also available on pages for specific conditions such as [Chiari malformation](#) and [hydrocephalus](#).

Print Materials and Information for UChicago Medicine Faculty and Staff

The program's team is also creating print materials to help patients and families learn about anomalies of the central nervous system. These materials will be available through the clinic, program staff, and available on the MHFP website. In addition, faculty and staff at UChicago Medicine will receive documentation about the MHFP. These internal documents will inform nurses, call center staff, and physicians about the MHFP and will provide guidance about how and when to refer patients to the program.

Program Staffing

We continue to build staff support for the MHFP. In June 2019, Rosa Velazquez joined the team as the program administrator and patient advocate. In recent months, she has focused primarily on coordinating approval processes that will allow for data sharing among collaborating institutions.

Judith (Judie) Holleman officially joined the MHFP team as an advanced practice nurse. She will support patients and help advance research initiatives, as described in the following section of this update. Holleman is currently a pediatric nurse practitioner in pediatric neurosurgery at UChicago Medicine. She brings a wealth of experience and a strong commitment to caring for patients impacted by CNS anomalies.

Currently, the team is in the process of recruiting a masters-degree-level program manager to handle administrative oversight of the MHFP. The program manager will coordinate operations and communications activities and will serve as a liaison between various stakeholders. The team is also working to engage two Child Life Specialists, who each will dedicate 30 percent of their time to working with MHFP patients and families.

Over the coming year, the MHFP team plans to build on these accomplishments by achieving the following objectives in the area of patient-focused outreach and support:

- Develop and roll out online resources that help patients navigate care and understand conditions and treatment options. These resources will be available via the MHFP website and will be distributed directly to patients in the clinic.
- Engage Comer Children's Hospital Patient Facilitators who will provide guidance and support to patients and their families as they navigate the various treatment options and resources. The patient facilitators will work with patients throughout their care. Judie Holleman will train these individuals on working with CNS patients and will support activities in the hospital and beyond, including group gatherings for patients and their families.

- Continue to build relationships with organizations such as Bobby Jones CSF, Conquer Chiari, and the Illinois Spina Bifida Association to establish a network that will help patients more easily access resources and support services and connect with specialists at UChicago Medicine.
- Recruit a full-time program manager to oversee and ensure the implementation of internal and external MHFP initiatives, including operations, communications, and coordination among personnel.
- Explore options for hosting community gatherings, which would initially take place online. Events could include a *presentation and Q&A with doctors or a patient and family reunion*.

Pillar 2: Clinical Research

Advancing Multi-Institutional Collaborative Research

Clinical Data Gathering Consortium

The MHFP team coordinates a Clinical Data Gathering Consortium that includes physicians from around the country who are working to advance the treatment of congenital anomalies of the nervous system. Consortium membership currently includes:

- Carolina Neurosurgery and Spine Associates; Sarah Jernigan, MD, MPH
- Cedars-Sinai Medical Center, Los Angeles; Moise Danielpour, MD
- Boston Children's Hospital; Lissa Baird, MD
- University of Chicago Medicine; David Frim, MD, PhD

Lissa Baird, MD, was formerly the consortium representative from Oregon Health and Science University. She remains involved in her new role at Boston Children's Hospital. The MHFP team plans to reach out to another faculty member at Oregon Health and Science University, and they hope to confirm that institution's continued participation in the consortium.

Consortium members are currently working on completing data-sharing agreements that will allow them to enter patient data into the multi-institutional database housed at UChicago. They are also working together to define priority research questions focused on improving patient care.

The Annual MHFP Symposium

The second annual MHFP Symposium took place as an online event on May 29, 2020. The symposium brought experts from around the county together to discuss the latest developments in the treatment and understanding of CNS anomalies. A total of fifty-five individuals attended the virtual event, representing facilities including: the University of Chicago, the University of Illinois at Chicago (UIC), Cedars-Sinai Medical Center/UCLA, Advocate Lutheran General Hospital, La Rabida Children's Hospital, Pepperdine University, and the University of Akron. International participants from Indonesia, Ecuador, and Bulgaria have also viewed the webinar.

The symposium covered a variety of central nervous system congenital anomalies. It featured presentations by the following experts:

- David Frim, MD, University of Chicago Medicine
- Judith Holleman, APN, University of Chicago Medicine
- Carina Yang, MD, University of Chicago Medicine
- Lissa Baird, MD, Boston Children's Hospital
- Moise Danielpour, MD, Cedars-Sinai Medical Center
- Francis Loth, PhD, University of Akron

During the event, speakers explored specific congenital conditions in detail and discussed the most advanced treatment approaches. Participants learned about models of care and innovations that could improve treatment in the future.

After this event, participants provided feedback through a survey. The majority of participants rated the webinar as excellent, and 88 percent said they plan to implement changes to their practice based on what they had learned.

Year 3 Activities

In Year 3, the MHFP team plans to take the following steps to continue expanding the consortium and facilitate collaboration among members.

- Identify data of interest to explore, with the goal of submitting a manuscript for publication by June 30, 2021.
- Incorporate one or two additional members into the consortium.
- Explore the possibility of hosting additional online events for physicians and researchers, such as a panel discussion or debate featuring leading experts

Creating a Comprehensive Database

Database Construction and Data Gathering

The MHFP team continues to make progress in constructing a comprehensive, multi-institutional patient database for the study of CNS congenital anomalies.

Christine Herrera joined the team in Year 2 in the role of clinical research coordinator, and she continues to oversee database maintenance and management of two databases. The two databases associated with this project are:

- The **patient database**, which contains basic information about UChicago Medicine patients treated for CNS anomalies. This database now includes patient information for approximately 10,000 individuals treated since 1995.
- The **MHFP Research Electronic Data Capture (REDCap) database**, which is the comprehensive clinical database that will include information from all consortium members and will be accessible to all member institutions. The database will consist of extensive, de-identified information about patients. This means investigators will be able to access the information required to conduct research, but they will not be able to identify individual patients treated at other institutions.

Data-Sharing Approvals and Data Entry

Early in Year 3, the University of Chicago's Internal Review Board (IRB) approved clinical data collection and the creation of the MHFP REDCap database. This approval for comprehensive clinical data collection for both retrospective and prospective patient cases ensures that the MHFP will be able to house the research database containing extensive clinical information.

The IRB also approved a set of templates that will establish specific data fields and ensure that data collection processes are consistent across institutions. Templates have been completed and approved for Chiari malformation, hydrocephalus, tethered cord syndrome, and achondroplasia.

With these approvals in place, the MHFP team has started to transfer information from the patient database to the REDCap database. They are also working on obtaining the approvals needed to enter data from partner institutions.

Year 3 Activities

In Year 3, the team plans to reach the following milestones in their efforts to advance the construction and utilization of the database:

- Use the guidelines and templates created to enter retrospective patient information from UCM and consortium members into the central data repository.
- Obtain approvals needed to add data from other consortium member institutions to the REDCap database.
- Once retrospective data have been collected and entered into the database, continue to add information about new cases and create templates for additional conditions.

Advancing Clinical Research at UChicago Medicine

The Clinical Research Team

Dr. Frim leads UChicago Medicine's clinical research on CNS congenital anomalies. The following UChicago faculty and staff members are also contributing to these research endeavors.

- Neuropsychologist **Maureen Lacy, PhD** is working to unravel the cognitive impacts of hydrocephalus and Chiari malformation type 1.
- Neuroradiologist **Carina Yang, MD**, is evaluating past approaches to treatment for CNS congenital anomalies and advising on the selection of imaging modalities for use in innovative clinical trials.
- Student research assistant **Abigail Herman** is working with **Christine Herrera** to organize and enter data for the REDCap database.
- Starting in October, **Judith (Judie) Holleman, APN**, will join the team as an advanced practice research nurse. She will split time between front-facing clinical efforts and research activities such as developing clinical research protocols and helping administer clinical trials. She will work closely with congenital anomaly patient families, who will benefit greatly from having an experienced clinician help interpret and navigate the pathway of care.

Current and Upcoming Investigations

Dr. Frim is in the process of writing a paper on Chiari malformation and hearing and expects to submit it for publication during year 3. Dr. Frim is also interested in studying the impact of the COVID-19 pandemic on individuals with CNS congenital anomalies. This could involve surveying patients and families during clinic visits to learn how the pandemic is impacting their lives.

Year 3 Activities

Research goals for Year 3 include the following.

- Initiate at least two investigations that utilize the patient database to conduct translational research.
- Submit at least two studies for publication in peer-reviewed journals.
- Complete the process of onboarding Judie Holleman in the role of advanced practice nurse.

Pillar 3: Basic Science/Laboratory Research

Dr. Frim and his colleagues are moving forward in their efforts to advance our basic scientific understanding of CNS congenital anomalies.

Laboratory Staffing

This fall, the team will onboard a research professional to support Dr. Frim's lab. They have hired Renuka Ramachandra, PhD, to fill this role. Dr. Ramachandra is a neuroscientist with over 19 years of experience in finding molecular pathways, drug discovery, product design and validation, stem cell therapy, and testing for conditions such as Parkinson's disease, stroke, spinal cord injury and pain. She is passionate about the neurological sciences, and her expertise and experience will contribute greatly to the MHFP research mission. Dr. Ramachandra will help advance multiple investigations, starting with supporting Dr. Frim's research on Chiari malformation and brain injury.

Collaboration with the University of Illinois at Chicago

Dr. Frim has been collaborating with Andreas Linninger, PhD, at the University of Illinois at Chicago (UIC) on basic science initiatives. Dr. Linninger is conducting research aimed at improving hydrocephalus shunting devices and pressure-sensing devices. This work could lead to better outcomes for patients with cerebrospinal fluid shunts. In the past six months, Dr. Linninger has identified a post-doctoral researcher to support his work. He is in the process of hiring Seifollah Gholampour, PhD, an international hire who is currently in the process of obtaining his visa that will allow him to come to Chicago.

Collaboration with the University of Akron

This year, Dr. Frim and his team have also continued to collaborate with Francis Loth, PhD, at the University of Akron. Dr. Loth is the creator and executive director of the Conquer Chiari Research Center at the University of Akron. He is investigating possible connections between subtle variations in the anatomy of the skull and the presence or absence of symptoms in patients with Chiari malformation type 1. Dr. Frim's team is currently utilizing the MHFP databases to provide data to assist in this research by. To date, they have provided data and imaging for approximately one third of the 100 Chiari patients needed for Dr. Loth's portion of the study. Data collection should be completed by December.

Year 3 Activities

Year 3 goals in the area of basic science are:

- Fully onboard Renuka Ramachandra to support Dr. Frim's research lab.
- Fully onboard a post-doctoral fellow to support Dr. Linninger's work at UIC.
- Complete the research study with Dr. Loth by providing 100 patient cases to Dr. Loth for analysis.

MHFP Team Members

University of Chicago Medicine

- **David Frim, MD, PhD**, lead investigator
- **Ryan Cyzman**, operations manager
- **Abigail Herman**, student research assistant
- **Christine Herrera**, clinical research data manager
- **Judith Holleman, MSN**, advanced practice nurse
- **Maureen Lacy, PhD**, neuropsychologist
- **Renuka Ramachandra, PhD**, research professional (starting November 2020)
- **Rosa Velazquez**, program administrator and patient advocate
- **Carina Yang, MD**, neuroradiologist

Researchers at Collaborating Institutions

- **Lissa Baird, MD**, Boston Children's Hospital, consortium member
- **Moise Danielpour, MD**, Cedars-Sinai Medical Center, Los Angeles, consortium member
- **Sarah Jernigan, MD, MPH**, Carolina Neurosurgery and Spine Associates, consortium member
- **Andreas Linninger, PhD**, University of Illinois at Chicago, collaborating researcher
- **Francis Loth, PhD**, University of Akron, collaborating researcher

Thank You

Because of your partnership, researchers at the University of Chicago and collaborating institutions are making important advances that will improve the lives of people impacted by CNS congenital anomalies. We are truly grateful for your support and looking forward to keeping you informed about the progress we make in the coming months and years.