



AT THE FOREFRONT

UChicago
Medicine



MARGARET
HACKETT
FAMILY CENTER

Improving Care for Central Nervous System Congenital Anomalies

Margaret Hackett Family Center Progress Report

December 2019

Thanks to your generous support, the University of Chicago has established the Margaret Hackett Family Center (MHFC) as a hub for patient care and research aimed at improving outcomes and quality of life for people with central nervous system (CNS) congenital anomalies. Since the program was established last year, our faculty and staff have made meaningful strides toward achieving key goals, including building a warm and compassionate destination for care, organizing a research consortium, and constructing a database of knowledge to support clinical and translational studies.

Pillar 1: Patient Focused Outreach and Support

This year, key staff members were brought on board to support program initiatives focused on patient care. Rosa Velasquez was hired as patient advocate/program administrator in June 2019. In this role, she helps patients and families navigate information, resources, and treatment options related to CNS congenital anomalies.

In addition, Ms. Velasquez has begun to identify third party organizations that support patients and families living with CNS congenital anomalies. She plans to reach out to these groups to build a network of organizations that provides referrals to UChicago Medicine and offers additional support services to patients and their families. Ms. Velasquez has also initiated the process of creating web content about the MHFC, ensuring that patients will be able to easily locate information about the program through the UChicago Medicine website.

In order to increase the visibility of the MHFC, the UChicago Medicine development team wrote an article about the program and published the story on the UChicago *Give to Medicine* website in May 2019. The story was also featured in the Comer Children's newsletter, *Give Kids Health*, in June 2019. This newsletter is distributed to more than 8,000 pediatric donors and potential donors.

In the coming year, the MHFC team plans to build on these accomplishments by achieving the following objectives in the area of patient focused outreach and support.

- By the end of November 2019, publish content about the MHFC on the UChicago Medicine website and ensure that patients can easily navigate to this information by searching for relevant conditions.
- By February 2020, engage two part-time Comer Children's Hospital Child Life specialists as patient facilitators who will provide guidance and support to patients and their families as they navigate the various treatment options and resources. These patient facilitators will work with patients throughout their care. These individuals will be trained on working with

CNS patients and will support activities in the hospital and beyond, including group gatherings for patients and their families.

- Publish an article about the MHFC in fall issue of UChicago Medicine's *At the Forefront* magazine, which goes to more than 300,000 patients, family members, and others.
- Include information about the MHFC in the Department of Surgery's annual report, to be released in the Fall/Winter of 2019.
- Develop relationships with 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.

Pillar 2: Clinical Research

Establishing a National Consortium

Over the past year, the MHFC team organized a Clinical Data Gathering Consortium. Its members include physicians from around the country who are committed to the care and study of patients with 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
- Oregon Health and Science University; Lissa Baird, MD
- University of Chicago Medicine; David Frim, MD, PhD

UChicago Medicine hosted the first annual MHFC Symposium on June 21, 2019. Since then, the four consortium members have met three times and will continue to do so every two weeks. In their meeting on October 11, 2019, the group discussed guidelines for data entry and sharing.

Consortium members will continue to work together to pool data and improve patient care. Each site is conducting a retrospective review of their CNS anomaly patient cases and will share specific data points with UChicago Medicine for compilation and further analysis.

Over the coming year, the MHFC team plans to take the following steps to continue expanding the consortium and facilitate collaboration among members.

- Identify 2 data points to be analyzed as a group.
- Identify 2 additional consortium members by the end of year two, such that they are incorporated into the consortium at the beginning of Year 3.
- Hold the second annual MHFC Symposium in June 2020.

Creating a Comprehensive Database

Progress is also underway in an effort to build a comprehensive, multi-institutional patient database for the study of CNS congenital anomalies.

In September 2019, Christine Rodriguez was brought on board in the role of clinical research data manager. She will be responsible for the construction, maintenance, and management of the database. She will work with researchers to develop standard protocols for data collection and data entry, and will support researchers in utilizing the database for their investigations.

Student research assistant Abigail Herman joined the team in July 2018, and has been helping with the initial collection of clinical data. In the coming months, the team will hire an additional student employee who will also assist with these efforts.

With data coming in and the research data manager in place, the consortium team has started the processes of consolidating and standardizing patient information. They are developing templates for each condition, which will establish standard data fields and ensure that data collection processes are consistent across the consortium.

Over the coming year, the team plans to reach the following milestones in their efforts to advance the construction and utilization of the database:

- Finalize database templates by the end of December 2019.
- In January 2020, start entering patient information from UChicago and consortium members into the central data repository, starting with retrospective data.
- Once retrospective data has been collected and entered into the database, continue to add information about new cases.

Advancing Research

Dr. Frim leads the UChicago team's clinical research efforts. Additional collaborators who have joined the program team include the following faculty and staff members:

- Statistician **Ying Cao** is analyzing data from UChicago and consortium members.
- 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.

Several research initiatives are already underway. Dr. Frim is working with Maureen Lacy, PhD, in behavioral neurosciences and surgery to complete a paper that is expected to be published within the coming year.

In addition, Dr. Frim is working on with Ying Cao and Dr. Yang on a project that they expect to submit for publication in the coming year.

Research goals for the coming year include the following:

- Identify at least two opportunities to use the patient database to conduct translational research.
- Submit at least two studies for publication in peer reviewed journals.
- By January 2020, hire an advanced practice nurse who will provide personalized care for patients and help administer clinical trials.

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.

David Wright, PhD, supported MHFC basic science initiatives up until his retirement in January 2019. Dr. Frim is currently in the process of recruiting a post-doctoral fellow to fill this role. The fellow will assist in basic science laboratory studies aimed at developing a model for brain injury that will simulate the types of brain injury and trauma seen in hydrocephalus patients.

In addition, Dr. Frim is collaborating with Andreas Linninger, PhD, at the University of Illinois at Chicago (UIC). 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.

Goals for the coming year in the area of basic science are:

- By March 2020, hire a post-doctoral fellow to support Dr. Frim's basic science research.
- By July 2, 2020, add a post-doctoral fellow to support Dr. Linninger's work at UIC.

Thank You

Thanks to your partnership, researchers at the University of Chicago and collaborating institutions are making important advances that will help to 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 coming months and years.