

Families Helping Families: A Report of Gratitude Prepared for the Firefly Fund

Thank You

Every gift to Rush University Medical Center advances our mission to improve the health of the individuals and diverse communities we serve. **Few gifts, however, touch the patients we serve as directly as your commitment to the Pediatric Neurology Rare Disease Patient Access Fund, or, as it's more informally known, *Families Helping Families*.** With your support of this fund under the direction of Dr. Berry-Kravis, lower-income families of children with rare diseases such as Niemann-Pick disease type C, or NPC, are able to begin receiving the most advanced care available — right away — for their child.

As you know from your firsthand experience, Dr. Berry-Kravis is one of just a few experts in the nation leading the most promising clinical trials of experimental therapies for NPC and other rare neurogenetic and neurodevelopmental diseases. Her team therefore treats families from all over the country fortunate to have found their way to her clinic at Rush. Regretfully, some families face additional barriers, particularly those with limited financial resources who are traveling great distances for their treatment and do not have insurance support for care. **Your support of Families Helping Families makes a truly life-altering difference for these families.** On their behalf and ours, thank you for your generosity. We hope to convey the profound impact of your giving in the following update.



“The fund does not support a lot of patients, but it allows us to treat specific patients who otherwise will not be able to get treatment and will permanently lose function and even die if we don’t treat them. The fund also allows us to start treating immediately without waiting the many months it will take to get state Medicaid insurance approval while the patient loses skills and neurological function forever.”

- Dr. Berry-Kravis

Your Gift: A Catalyst for Improving Our Policies at Rush

Following your initial gift to establish Families Helping Families, Rush leaders needed to determine a process for operationalizing and administering these funds to directly support Dr. Berry-Kravis’s patients in need. Developing this process unearthed a well-intended but problematic flaw in Rush’s policies. Rooted in Rush’s community-minded mission — and to prevent exploitation from any opportunistic individuals out of Rush’s primary service area — a policy was established long ago to prevent the use of philanthropic “charity care” funds for patients living beyond 50 miles of Rush. For Dr. Berry-Kravis’s highly unique rare disease clinic, where families are frequently traveling from around the country and even other countries, this presented an obvious challenge.

Working with finance leaders, Dr. Berry-Kravis and colleagues were successfully able to create an exception to Rush’s charity care policy for patients with rare diseases. Understanding that specialists in these conditions (not to mention clinical trials of

promising therapies) are not widely available, Rush leadership amended this policy for orphan diseases for the future – a critically important outcome of your gift that will make a positive difference for future patients and families under the care of Dr. Berry-Kravis and other physicians with rare disease expertise.

Four Families Forever Grateful for Your Generosity

Highlighted below are four patients who have been direct beneficiaries of Families Helping Families. Each of these children has a diagnosis of NPC and had to access treatment through expanded access for a drug called VTS-270/adrabetaDEX, which is in development by Mallinckrodt Pharmaceuticals, or MNK. At the time Rush was the only site in the U.S. these patients could access treatment from almost immediately. Here, with the support of your generous gift, Dr. Berry-Kravis is able to quickly initiate treatment through expanded access (sometimes referred to as “compassionate use”) for eligible patients. For families who come from far distances, Dr. Berry-Kravis can begin the protocol, stabilize the patients, and then sometimes partner with specialists closer to the family’s home to continue treatments over the longer-term. This process can often take several months to a year, however, with families coming back-and-forth between Rush and their hometowns in the interim. **Your gift lends a critical hand of support to these traveling families, offsetting their medical expenses.**

While MNK provides the drug free of charge in expanded access, medical expenses needed to administer the drug are not covered and must be billed to the family’s insurance. Unfortunately, Rush cannot typically bill uninsured or underinsured families who come from out-of-state. Many of these families have highly restrictive Medicaid managed care plans that are only accepted by providers closer to their homes. **Philanthropic support eliminates these gaps in coverage and helps offset out-of-pocket treatment costs these families would otherwise face.**

- An 11-year-old girl came from Kentucky to obtain treatment through expanded access and was treated by Dr. Berry-Kravis at Rush for four months before she was able to enter into an MNK trial and ultimately transfer to a Nashville hospital closer to her family’s home where she could continue her care. According to Dr. Berry-Kravis, “We wanted to start her treatment right away because she was losing skills due to NPC. She has done well and was stabilized for at least a year.”
- A 2-year-old boy came to Dr. Berry-Kravis from Ohio. He experienced developmental delays in motor and language skills due to NPC, and the private insurance his family had denied his care at Rush because it was an out-of-state facility. “He was treated as soon as we saw him with stabilization and developmental progress in many areas,” Dr. Berry-Kravis reports. He has now transferred to a medical center much closer to his home in Akron where he can be treated in-state to continue his specialized care.
- A 3-year-old boy came from Mississippi to Rush last year with a Medicaid plan Rush could not use and was rapidly declining due to NPC. According to Dr. Berry-Kravis, “He has been mostly stable since starting treatment and has even made developmental progress in certain areas.” Although he was briefly transferred to an Alabama medical center, he is back at Rush indefinitely to continue his treatments here until a location closer to his home can be found.

- An 8 year old patient with NPC living in the USA after returning from India to access treatment was able to get necessary CPAP equipment through the fund for hospital discharge to live near Rush and continue her treatment.

The Continued Need

Dr. Berry-Kravis anticipates utilizing the Families Helping Families fund to support one patient traveling to Rush from the Netherlands that will need to be treated through expanded access until he has health insurance in the U.S. Of course, several other families of children with NPC seeking Dr. Berry-Kravis's expertise could benefit from this fund, which is reserved for the most exceptional circumstances. Until there is a systematic change to fight health inequities or a cure for this disease, Dr. Berry-Kravis and the philanthropy team at Rush will continue to seek support for this fund from grateful patient families and other philanthropic sources. Maintaining a balance in this fund is critical so that Dr. Berry-Kravis can quickly access needed resources to support families when emergencies arise. With the impending closure of the MNK trials and all patients in the U.S. moving to treatment through expanded access, it is expected the importance of funds such as this will only grow.

In Gratitude

We extend our profound gratitude for your gifts to the Rush University Children's Hospital and Families Helping Families. We hope this report demonstrates the direct and life-altering impact your support has on the families who turn to Dr. Berry-Kravis's team for their care and are eager to discuss the power of your giving in greater detail. Once again, thank you for your commitment to this initiative.

APPENDIX A
EXPENSED ITEMS FROM PHILANTHROPIC FUND

Your gift, and those of other donors, provided the critical resources needed to support clinical care for 4 patients, that otherwise would not have been accessible. We still have one additional patient that we would like to enroll through the Families Helping Families fund. Below is the estimated* breakdown of the philanthropic dollars spent to date.

*We are still reconciling the final dollars for anesthesia

Description	Amount
Lumbar Spinal Puncture	\$788.00
Therapeutic Spinal Puncture	\$6,087.16
Fibro Scan Testing	\$42.00
New/Return Patient Clinical Visits	\$6,131,79
Anesthesia	\$22,509.00*
Total Home Health – CPAP/Breathing Machine while in Chicago	\$1,600.00
Total Charges	\$37,157.95
Firefly Fund Philanthropy	\$25,000.00
Additional Donor Contributions	\$3,212.50
Total Philanthropy	\$28,312.50