FUNDING APPLICATION

GENERAL INFORMATION

Organization Information

Legal Name:	Federal Tax ID#:	501(c)(3) Public Charity
Columbus Children's Foundation	83-1456169	509 (a)(2)

Address:	City:	State:	Zip Code:
870 Martin Luther King, Jr. Blvd.	Chapel Hill	NC	27514

Website:	Fax:	United Way Funded:
columbuschildren.org	(612) 437-8836	No

Fiscal Year:

July 01 to June 30

Head Of Organization

Name:	Title:
Laura Hameed	Executive Director
E-Mail Address:	**Phone:**
lhameed@columbuschildren.org	(612) 437-8836

Application Contact

Name:	Title:	E-Mail Address:	Phone:
Elisabeth Krimbill	Dr.	ekrimbill@att.net	(210) 273-5099

Has the organization applied to the Najim Family Foundation in the past and been declined?

No

Grant Amount Requested $:	Total Project Budget $:	Organization's Annual budget $:
$50,000	$250,000	$20,000,000

Mission Statement:

The Columbus Children's Foundation is driven by the urgency to accelerate equitable access to the most effective gene therapy solutions for children with ultra-rare genetic diseases.

PROJECT INFORMATION

Program / Project Title:

To support the 2020 Parent and Patient Advocacy Boot Camp in Houston, Texas for families of/and children with ultra-rare diseases.

PROJECT TIMELINE

Start Date	End Date
06/01/2020	05/31/2021

Harvey E. Najim Family Foundation Priorities:

Medical Needs

Program / Project Description:

Hope Accelerates Here

An Ultra-rare disease is defined as a condition that affects less than one in 50,000 people worldwide and one out of every two people diagnosed with a rare disease are children. Many of these rare diseases are 1 in a million diagnosed in fewer than 500 children worldwide. And some, like Juvenile Parkinson's Disease, are even more rare and affect only one in 54 million, impacting approximately 130 children worldwide. And, while these diseases are ultra-rare, these children should not be ignored by science or denied the hope for a curative solution.

Sadly, since the number of children with these diseases is so low, far too often, traditional commercial research for cures is not adequately funded or developed leading to an innovation gap for patients. These are referred to as "ultra-orphan" diseases. As the pharmaceutical industry mainly focuses on more common illnesses with greater commercial potential, these ultra-orphan diseases are often overlooked or unable tomove forward even when promising data exists.

We believe innovation should not ignore ultra-rare diseases. The Columbus Children's Foundation is prepared to bridge the innovation gap. We believe no matter how rare a disease is, if there is a promising path to innovation and curative solutions someone needs to light the way for these patients with life threatening ultra-rare genetic diseases.

One disease at a time. One child at a time.

A Unique Approach to Save Lives

The Columbus Children's Foundation has developed a non-profit development model focused on advancing and accelerating promising gene therapy treatments into the clinic. This model is squarely focused on meeting significant unmet medical needs that are not currently being met in the market, rather than a focus on driving return on investment. Through this model, we bring together industry, philanthropy and research partners to drive innovation for ultra-rare diseases across all disease platforms where gene therapy shows promise.

Additionally, we are committed to sharing data from our programs into the public domain (where practicable) to help build and accelerate scientific knowledge and propel the entire gene therapy community toward advancing treatments. We believe our Juvenile Parkinson's data has the potential to advance understanding of adult Parkinson's Disease on a much broader scale.

Your support will help shape the future of genetic medicine and will save lives.

Designing a Roadmap for Parents and Patient Advocacy Organizations

As we seek to bring cures to children with ultra-rare diseases, we also know that the journey families must navigate to support finding the cure for their child born with an ultra-rare genetic disease must be improved in order to accelerate equitable access to cures in time for their child. Three out of eight affected children will not see their 5th birthday... unless, we can accelerate cures and bring hope to families.

When a family gets a diagnosis for their child with an ultra-rare disease, they embark on a desperate mission to find a cure in time. Many parents will join or start a patient advocacy foundation in support of their child's specific disease. This heartbreaking process is reinvented over and over for these families in need of support. We can help support this process and help families make better sense out of a complex path filled with dead ends and frustration as they try to navigate a deeply scientific world. We have developed a Parent and Patient Advocacy Boot Camp to bring together experts, train attendees to be effective communicators for their disease program, and connect them to the components of a quicker roadmap to find a cure for an ultra-rare disease.

Topics included in the training:

o Taking action after diagnosis.

o Communicating effectively with stakeholders

o Engaging others to create a patient advocacy organization

o Work with researchers to advance a cure.

o Understanding regulations regarding clinical trials

o Processes and procedures regarding manufacturing a cure

o Protecting the intellectual property to ensure equitable access

o Care for the Caregiver

Our Boot Camps will arm parents and patient advocacy organizations with the tools to traverse the complex path to a cure with confidence. Your contribution will support these programs to accelerate cures and save lives.

We have scheduled 5 Boot Camps around the country. One of the 2020 Boot Camps will be held in Houston, Texas. Texas is recognized internationally for its strong scientific community which is leading the way in gene therapy solutions for ultra-rare diseases. Each Boot Camps will serve 20 parent/patient advocacy groups representing approximately 100 kids per group desperately in need of a cure. Your support of one Boot Camp has the potential to impact the lives of over 2,000 children living with devastating and life-threatening, ultra-rare diseases. Nationwide, our Parent and Patient Advocacy Boot Camp program has the potential to impact the lives of over 10,000 of these very special children and their families.

With the Support of the Harvey Najim Foundation, We Will:

o Support the development of curative gene therapy solutions for children with ultra-rare and neglected diseases.

o Aspire to advance and support outcomes that aim to transform the lives of these critically ill children.

o Accelerate access to innovative therapies for rare pediatric neurodegenerative diseases through our efforts both internally in our programs and externally in our partnerships we support and facilitate.

o Develop relationships with and engage industry experts to ensure the best minds are at the table and available to assist in acceleration and advancement of therapeutic options for children with ultra-rare diseases.

o Offer 5 Parent and Patient Advocacy Boot Camps nationwide.

Hope Accelerates Here with Your Support!

Evaluation Plan:

We recently opened a fundraising campaign to support our Parent and Patient Advocacy Boot Camps and programs related to accelerating cures for ultra-rare genetic diseases. This campaign aims to raise $250,000 to provide our first five Boot Camps cost-free to the participants throughout the United States.
Our participants will complete pre- and post-Boot Camp assessments to evaluate topics of focus including: taking action after diagnosis, communicating effectively with stakeholders, engaging others to create a patient advocacy organization, working with researchers to advance a cure, understanding regulations regarding clinical trials, processes and procedures regarding manufacturing a cure, and protecting the intellectual property to ensure equitable access.
The pre- and post-Boot Camp assessments will be analyzed and evaluated informing future presentations and guide adjustments to best meet the needs of the parents and patient advocacy groups. The surveys will be conducted through google forms and analyzed for determining best practices and impact.

Plans to sustain project beyond the term of this request:

We are excited to include the Harvey Najim Charitable Foundation as part of our initial collaborative team of supporters dedicated to funding these vital Parent and Patient Advocacy Boot Camps. With intentionality, we have apportioned the cost of the national and potential international camps into financially viable components.
In the future we plan to offer at least five Parent and Patient Advocacy Boot Camps each year to support our families and reach out to new families who have recently faced the devastating news that their child has been diagnosed with an ultra-rare disease.
This grant brings the Harvey Najim Charitable Foundation into our research consortium on the ground level. Together we can create a future in which all children diagnosed with an ultra-rare genetic disease can have equitable access to the treatments that will save their lives.

Children Impacted:

How many unduplicated children will the TOTAL PROJECT INITIATIVE impact?	**How many unduplicated children will NFF REQUESTED FUNDS impact?**
10,000	2,000

Please provide the percentage of each group below that will be served by the project in which funds are being requested. Do not leave any area blank. If that specific group will not be served, include zero. The percentage should total 100%.

A. Population Served Age		**B. Population Served Ethnicity**	
Infants (0-5)	80%	African American	10%
Children (6-13)	20%	Asian American	10%
Young Adults (14-18)	0%	Caucasian	50%
TOTAL:	**100%**	Hispanic/Latino	30%
		Native American	0%
		Other and Define	0%
		TOTAL:	**100%**

City Council District for Which Children are Being Served:

Outside San Antonio

Counties applicant is in:

Atascosa, Bandera, Bexar, Comal, Guadalupe, Kendall, Kerr, Medina, Wilson

Line item Budget:

Line Item Description	**Total Project Funds Allocation**	**Najim Funds Allocation**
Parent and Patient Advocacy Boot Camp	$250,000	$50,000
TOTAL:	**$250,000**	**$50,000**

OTHER FUNDING RESOURCES

For Project being Requested: Funding sources and amounts, pending and committed.

PROJECT - PENDING

Funder Name	**Amount Requested**
	$0
TOTAL:	**$0**

PROJECT - COMMITTED

Funder Name	**Amount Requested**

	$0
TOTAL:	**$0**

Other funding sources and amounts, pending and committed not specific to this request.	
ALL OTHER ORGANIZATION REQUESTS - PENDING	
Funder Name	**Amount Requested**
Ultragenyx	$50,000
Asklepios Bio	$75,000
Global Genes	$12,500
Rare Disease Council	$12,500
Termeer Foundation	$50,000
TOTAL:	**$200,000**
ALL OTHER ORGANIZATION REQUESTS - COMMITTED	
Funder Name	**Amount Requested**
	$0
TOTAL:	**$0**

BOARD OF DIRECTORS

What percentage of your board contributes financially to the organization?

100%

If Board giving is not at 100%, please explain why?

How are board members expected to participate in your organization?

Most pharmaceutical and life science companies focus on genetic disorders with commercial potential thus, ultra-orphan diseases are often overlooked because the economics don't add up. Our aim is to provide equitable access to treatment and research so children with ultra-rare genetic diseases despite where they live and how much money their parents may have. Most of these children are suffering a severely altered way of life and, in many cases, their life expectancy is shorter than ten years.
Our board includes the researchers and physicians who are currently developing and bringing curative gene therapies to their young patients who desperately need them. Every member of our board supports the organization through financial donations, in addition to their significant contributions of time and their very specialized talents and technical knowledge. The trustees collaborate with research partners and clinical experts to advance ultra-rare curative gene therapies into clinics worldwide.

LIST OF BOARD DIRECTORS	
Name & Office Held	**Corporate Affiliation**
Dr. R. Jude Samulski, Ph.D.: Chairman and Chief Science Officer	Chief Science Officer of Asklepios Bio
Sheila Mikhail, J.D., MBA: Trustee	CEO of Asklepios Bio
Dr. Krystof Bankiewicz: M.D., Ph.D.: Trustee	Professor of Neurosurgery and Gilbert and Kathryn Mitchell Endowed Chair at The Ohio State University
Javier Garcia, MBA: Trustee	CEO of Viralgen
Dr. Damia Tormo, Ph.D.: Trustee	Partner in Columbus Venture Partners
Matt Kane, MBA: Trustee	CEO of Precision Biosciences
Robert Long: Trustee	Partner at Piedmont Capital Investments

Signature
Laura Hameed