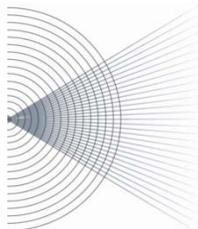


Newsletter of the Usher III Initiative

Winter 2012



USHER III INITIATIVE

DEDICATED TO DEVELOPING
CURES FOR BLINDNESS

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A Letter from Cindy Elden, Co-Founder, Usher III Initiative



Dear Friends,

Happy New Year and welcome to the third issue of the Usher III Initiative Newsletter! I am excited to share our news with you and I hope our progress-driven approach encourages you to get involved with our efforts.

We are pleased to announce the first EVER Spin-for-Sight, a fundraiser in New York City being organized by Rebecca Alexander and a small group of dedicated volunteers! All proceeds from this event will benefit our research and we hope you will consider becoming a part of this effort. Please see the Fundraising section below for details on how you can support our first Usher III Initiative Spin-for-Sight.

We have many developments on the research front to report. The Initiative is commencing “Proof of Concept” testing of several small molecule compounds. These tests will be a key factor in determining how to best move forward with a clinical trial for a treatment for Usher III. We hope to have initial results from this testing in time for our next research update meeting in early May. Research also continues at Case Western Reserve University and the University of Florida. Please read more on the progress of our dedicated team below in the Research Highlights section of this newsletter.

We continue to work with the Coalition for Usher Syndrome Research on the development of an Usher Syndrome registry. It is vital for patients to visit the site as soon as it is launched. This is not only important for us to identify patients for a potential clinical trial but it is very important for the steps leading up to any trial including providing demographic and other data needed for regulatory filings. The data collected will also aid us in obtaining available resources for a clinical trial. We will be sure to send out an alert as soon as the registry is launched.

In the meantime until the registry is launched, we cannot stress the importance of all Usher patients getting genotyped in order to find out which type of Usher Syndrome you have. This is absolutely critical and can be done with a simple blood test and without the registry. We encourage you to read the instructions below on how and where you can have your genotyping done. If you have only been clinically diagnosed (based on your symptoms), please read below and learn how you can get a genetic diagnosis. This is critical for you to be eligible for any upcoming treatments that may be available for any of the Usher Syndrome types.

If you haven't already joined us on Facebook, we encourage you to become a friend on the Usher III Initiative [Facebook](#) page today. While these newsletters are great ways to keep

To Support Usher III research...

The Usher III Initiative is 501(c)3 non-profit corporation. Donors can support our research efforts by making a gift via our website or by sending a check to our office. Gifts to the Usher III Initiative are tax deductible to the extent permitted by law.

For Online Donations

1. Go to our website at www.usheriii.org
1. Click on the link at the right side of our homepage under Donate "here" link
2. Complete the online form.

For Donations by Mail

Please write your check out to the Usher III Initiative and send your donation directly to us at the following address:

Usher III Initiative
191 N. Wacker Dr., Suite
2090
Chicago, IL 60606

shown that these two series may work with different mechanisms, offering the potential for more treatment options.

In keeping with the Usher III Initiative's multi-strategy approach to develop a treatment for Usher III, we continue pursuing gene therapy as a potential treatment. At the University of Florida, our gene therapy research continues as researchers are looking for safe and effective ways to deliver normal Clarin1 genes to the retina. These researchers are also searching for a 'phenotype' or surrogate marker of how effective the gene therapy may ultimately be. In addition to leading to a potential gene therapy treatment for Usher III, this 'phenotype' potentially can be used to gauge the effectiveness of our small molecule therapies as well.

Fundraising is in full swing!

We want to thank those of you who have supported our efforts in the past. We hope that this newsletter shows you how far we have come and in such a short time. We are determined to achieve our goal of developing a treatment in the fastest, most efficient way possible.

As we embark on full scale pharmaceutical production in order to achieve our goal, it will require more resources than ever and we hope that you are inspired by how close we are to achieving success. We hope you will join us and be part of a cure to Usher III.

Please consider making donations directly to the Usher III Initiative. You can do so by visiting our website www.usheriii.org or sending checks to the following:

Usher III Initiative
191 N. Wacker Dr., Suite 2090
Chicago, IL 60606

We also invite you to support the inaugural Spin-for-Sight taking place on February 26, 2012 at the Sports Club LA in New York City. If you aren't able to join us in person, please consider supporting one of the 50 riders supporting our cause.

This event has been spearheaded by fellow Usher III patient and Spin Instructor Rebecca Alexander and a dedicated group of volunteers. It is sure to be lots of fun as Rebecca leads us in two hours of spinning with a break or two in the middle. It will also be a great opportunity to get together with other Usher III patients, share stories, and get fit! All bikes have been filled but if you would like to sponsor a rider, make a donation to support the event, or attend to support our riders, please visit the event website at www.usheriii.org/index.

Other ways to get involved!

The Usher III Initiative's top priority is to develop a treatment for patients with Usher III. Our goal is to begin clinical trials in less than 2 years. In order to achieve this goal, patients should begin to plan for these trials now. Because Usher III is an orphan disease which does not affect a large population, it is important that each and every patient join our efforts to prepare for clinical trials. Early gathering of patient information will put us in a strategically better position to get to trials faster.

We urge all Usher III patients to take the following steps to be the most proactive patient you can be:

1. **JOIN THE USHER SYNDROME REGISTRY** to be launched online shortly by the Coalition for Usher Syndrome Research. The patient registry is critically important to clinical trials. You can get more information at the Coalition's website: www.usher-syndrome.org.

