Holidays newsletter

BCM Families Foundation <info@bcmfamilies.org>

Thu 12/23/2021 3:29 PM

To: renata.sarno@hotmail.com <renata.sarno@hotmail.com>

BCM FAMILIES FOUNDATION
www.bcmfamilies.org

Dear BCMFF Friends and Families,

2021 is coming to an end, so we want to send you warmest wishes for a Happy Holiday!

During 2021, scientists worked to develop the best outcome measures to assess the level of success in an upcoming human clinical trial for BCM intravitreal gene therapy. Meanwhile Adverum Biotechnologies made impressive progress toward submission of an Investigational New Drug (IND), that, when approved, leads to a clinical trial. BCMFF collaborated both with scientists and with Adverum Biotechnologies, arranging, among other supports, for a grant to be sent to Dr. Samuel G. Jacobson of the University of Pennsylvania to find a color vision assessment tool for BCM volunteers who will receive BCM gene therapy during the human clinical trial. We are immensely pleased to share the letter below, sent to all families affected by BCM from our colleagues and friends at Adverum Biotechnologies.

We've kept in touch with all of you by organizing zoom meetings and continuing to work on the BCM Patient Registry. Other projects have started, including a video game dedicated to being a diagnostic tool for BCM, as well as another project to organize a European meeting in Venice, Italy on April 9, 2022. Several additional projects have been listed in a BCM Families Foundation Strategic Plan, that we will be reviewing at the beginning of 2022 for final approval.

Another significant topics, please read below about what Orphan Drug Designation means, because this will be important to know in the next few months.

Many good wishes for a wonderful holiday season to all of you!

BCMFF Board of Directors.

In the following:

1) Happy Holidays from Adverum Biotechnologies!
2) What an Orphan Drug Designation is by Kay McCrary
3) Calendar of upcoming Regional Zoom Meetings
4) ZOOM/MAGNIFY Your TV with AppleTV Streaming Box by Dean Monthei
5) Do something great: join the BCM Patient Registry
6) An invitation to all European families with BCM and to all our members: join us at the meeting in Mestre/Venice, Italy on April 9, 2022

ADVERUM

Happy Holidays from Adverum Biotechnologies!

Adverum is a clinical-stage gene therapy company targeting unmet medical needs in serious ocular and rare diseases. Adverum has been working diligently on developing a gene therapy for the treatment of Blue Cone Monochromacy. Our investigational therapy, ADVM-062, is designed to deliver a functional copy of the human L-opsin gene to the central retina following a single intravitreal injection.

Through the dedicated efforts of our talented scientists, Adverum recently completed the investigational new drug (IND)-enabling Toxicology study and is getting ready to submit the IND to regulators. A successful IND submission is a major step in getting this therapy to patients. Adverum is committed to the ongoing collaboration with BCMFF and the world class experts in the field. In 2022, our efforts will be focused on bringing these experts together to advise us on the clinical trial design.

On behalf of the entire Adverum team we wish everyone at the BCM Families Foundation, physicians, patients, and their families, a safe, healthy, and happy holidays.

Pallavi Sharma
ADVM-062 Program Lead, Adverum Biotechnologies Inc.

ORPHAN DRUG DESIGNATION
Certain drugs and treatments have been designated as special, as Orphan Drugs, the medicines used for the diagnosis, prevention, and treatment of rare diseases. Blue Cone Monochromacy (BCM) is a rare disease, affecting only 1 person in 100,000, so a medicine for BCM has excellent potential to be designated an Orphan Drug, giving helpful incentives to biotechnological companies working on orphan drugs. Such incentives are

necessary and extremely important because orphan drugs for rare disease are significantly less advantageous for a biotech or pharmaceutical company, given that the market for a RARE disease is very narrow but the investments to be made are very high. A biotech company working on a drug for a rare disease is able to submit an Orphan Drug Designation request to the European Medical Agency (EMA) in Europe and to the Food & Drug Administration (FDA) in the USA. However, the Build Back Better Act, passed by the U.S. House of Representatives November 19, 2021 includes, according to the National Organization for Rare Disorders (NORD), "devastating changes to the Orphan Drug Tax Credit (ODTC). In a bill that would otherwise do so much good for so many Americans, it is unconscionable that Congress would target this critical incentive that has, for decades, successfully driven the development of new cures and hope for the rare diseas community." (Press Release from NORD, November 19, 2021).

EMA:
In Europe, a disease is considered rare when it affects no more than 5 people per 10,000 inhabitants. Orphan drugs in the European Union must meet the following criteria:
1) must be indicated for a life-threatening or chronically debilitating condition;
2) must be indicated for a rare clinical condition, defined by a prevalence of no more than 5 subjects per every 10 thousand individuals, calculated at the level of the European Union;
3) no valid treatments must be available or, if treatments are already available, the new drug must represent a significant clinical benefit.
EMA grants to the company producing an Orphan Drug: 10-year exclusivity, financial assistance, tax benefits.

FDA (at present):
The FDA has the authority to grant orphan drug designation to a drug or biological product to prevent, diagnose, or treat a rare disease or condition.
FDA grants to the company producing an Orphan Drug: 7-year exclusivity, incentives, tax benefits.
https://www.fda.gov/industry/developing-products-rare-diseases-conditions/designating-orphan-product-drugs-and-biological-products

BCM Families Foundation supporters in the United States face the urgent need to join the thousands of NORD's Rare Action Network advocates by contacting our legislators to share our concerns that limiting the availability of the Orphan Drug Tax Credit would jeopardize the development of new therapies for rare diseases, including BCM! Urge your Senator to remove this harmful provision when the Build Back Better Act comes to the U.S. Senate for consideration.

Calendar of upcoming Regional Zoom Meetings

- Saturday January 15th 3pm local time UK - Organizers: Trudi and Laura
- Saturday January 29th at 10:30 AM EAST - US Southeast + Florida - Organizers: Kay and Barbara
- Saturday February 12th US Northeast + East Canada - Organizers: Marylee
- Saturday March 12th US Midwest + Texas - Organizers: Jason and John
- Saturday March 26th US West Coast + West Canada - Organizers: Nicole and Dean

ZOOM/MAGNIFY Your TV with AppleTV Streaming Box

I spend a lot of time watching YouTube videos on a TV but was frustrated with not being able to read the video titles when sitting 6 feet (2 meters) from my 77" TV.

Most cell phones, computer screens and tablets have a zoom accessibility feature. There are very few TVs that have built-in zoom features and the ones that do have severe limitations. AppleTV is a streaming box that has a zoom feature that can zoom in as much as 15 times (15X) and can connect to any TV with HDMI input. It has a YouTube app as well as many other streaming apps like Netflix, Amazon Prime, etc. In addition it has apps for looking at iPhone photo galleries and other media sources.

The AppleTV has many of the same accessibility features as iPhones including zoom, high contrast, color filters and voiceover (reads text aloud).

The AppleTV costs $200 US for the 4K 64GB version and $150 for the 32GB version. You turn on the zoom feature under the Accessibility settings menu. This allows not only YouTube to be magnified but anything being displayed by the AppleTV (menus, photos, movies, TV shows, games, etc.). Broadcast TV is also available on AppleTV in some regions (this can be tricky to set up and I am not using it).

The instructions for how to use zoom on the AppleTV are shown on the lower left of the Accessibility page where you turn the feature on. Instructions are also given in the video link below. It does take some practice to get used to the correct finger gestures.

Here is a link to a 6-minute video on how to use AppleTV zoom and voiceover:

https://www.youtube.com/watch?v=RLSTJah7_nc

Dean Monthei

What are you waiting for?
Join the BCMFF patients' registry!
www.BCMRegistry.org

Who can register ?
Adults and children affected by BCM may participate in the BCM Registry. You must have genetic confirmation of BCM (DNA Test Report).

An invitation to all European families with BCM and to all our members: join us at the meeting in Mestre / Venice, Italy on April 9, 2022

The BCMFF and the Italian Achromatopsia Association are pleased to invite families to the Meeting of Families Living with Achromatopsia and Blue Cone Monochromacy to be held on April 9, 2022 in Mestre-Venice, Italy. There will be scientific presentations and interactions between patients, scientists, pharmaceutical companies.
Although the meeting is in Europe, and we hope to arrange a meeting in North America soon, all members of the BCM Families Foundation are welcome to attend. A scholarship that covers hotel room expenses is available for families with BCM who cannot afford

travel expenses.

In this season of gifts, remember BCMFF

DONATE


mailchimp