

THE CURE CHRONICLES

August 2022 ISSUE

Enabling affordable and accessible advanced medicines

Greetings,

Welcome to the Hot Summer news editions of the Cure Chronicles! Thank you for your continued interest in our mission to bring affordable cell and gene therapies to all who would benefit. In this edition's Spotlight, we feature a business leader whose training, experience, and insight has led to a truly impactful career, **Dr. Mohammed Asmal**.

In our News section, we take the time to recognize the successful approval of a gene therapy for severe beta-thalassemia, an approval that has challenged other commercial developers, and renewed the resolve in the patient and advocacy community to find a way to bring down the cost of gene therapy. We also present some of the breakthrough news from the #IAS2022, The 24th International AIDS Conference, in Montreal, Canada.

News

Here is the latest on what we are excited about:

For those who tuned into the FDA Cell and Gene Therapy Advisory Committee meeting this June, it is no surprise that Zynteglo (beti-cel, betibeglogene autotemcel) has now earned marketing approval. The presentations in June to the FDA by Bluebird Bio, Inc., demonstrated a deep level of clinical, scientific, and regulatory excellence, resulting in an impactful new approach for patients. The presentations for a lentiviral

gene vector approach (eli-cel, elivaldogene autotemcel) for cerebral adrenoleukodystrophy (CALD) were equally impressive. In both cases the Advisory Committee's vote was unanimously in the affirmative.

Follow the link below for the August 17 press release from the FDA announcing the Zynteglo approval:

→ [FDA Approves First Cell-Based Gene Therapy to Treat Adult and Pediatric Patients with Beta-thalassemia Who Require Regular Blood Transfusions | FDA](#)

The following link to the Bluebird Bio press release references the clinical trials presented:

→ <https://investor.bluebirdbio.com/news-releases/news-release-details/fda-advisory-committee-unanimously-supports-beti-cel-gene#:~:text=bluebird%20bio%20is%20eligible%20to,potential%20approval%20of%20beti%2Dcel.&text=Beta%2Dthalassemia%20is%20a%20severe,or%20absent%20adult%20hemoglobin%20production>.

The International AIDS Conference in Montreal (sponsored by the International AIDS Society) was noted for both meaningful presentations, the global village presentations, and protests as to the inability of many international visitors to gain visas in time for the conference. The big clinical news was that another patient was found to have cleared viral infection following bone marrow transplantation for a hematologic malignancy. Timothy Ray Brown's wish to "not be the only one," is indeed proving to be true. However, this latest news (link below) demonstrates that principles that have now been to be established-must be expanded in a non-malignant treatment setting.

→ <https://www.medscape.com/viewarticle/978169>

→ <https://www.cityofhope.org/patient-achieves-hiv-and-blood-cancer-remission-three-decades-after-hiv-diagnosis-through-stem-cell>

A few members of the GGTI also gave a very impactful presentation on the need to develop and HIV Cure strategy. Caring Cross is a supporting member of the GGTI (Global Gene Therapy Initiative), and hosts an informational page where support can

be directed: <https://caringcross.org/global-gene-therapy-initiative/>

Dr. Jenifer Adair, Dr. Cissy Kityo, Michael Louella, Moses Supercharger and Dominic Kemps led a townhall style discussion at #IAS2022 titled, "AIDS 2022- On the road to HIV cure gene therapy: Who can learn from whom?" Grab a cup of tea, and enjoy listening to the discussion: <https://caringcross.org/presentations/>

Another core activity at Caring Cross is the creation of a set open protocols and readily available reagents for the productions of both lentiviral gene vectors (LV) and adeno-associated virus (AAV) vectors. This project will allow for broad standardization across the field and give a firm footing to academic and early start-up organizations to produce LV and AAV that will be compatible with future clinical development. The poster presented at the NIIMLB annual meeting describing our progress thus far is available for free download on the Caring Cross website.

➔ [**OPEN LENTIVIRAL VECTOR AND ADENOVIRAL-ASSOCIATED VIRAL VECTOR MANUFACTURING PLATFORMS \(ARP-27\)**](#)

Caring Cross is also excited to share that a new partnership with Cytiva has been formalized. Cytiva has recognized that the workflows Caring Cross is developing can have broad impact, especially in low- and middle-income countries where place-of-care manufacturing of cell and gene therapy could lower the cost sufficient to make HIV, sickle cell disease, and immuno-oncology CAR-T therapies all possible.

➔ [**Cytiva supports Caring Cross to develop CAR-T cell therapy for people with HIV in low-to-middle-income nations**](#)

Community Spotlight

Mohammed Asmal MD-PhD

Venture Partner, OrbiMed Advisors LLC



Tell us about your background and how you came to be in your current position.

I am a clinician-scientist, and drug developer in oncology, infectious diseases and gene therapy. Born in South Africa, and raised in Boston, I was shaped by the crises faced in my homeland and the opportunities afforded me by my adopted home. During the time I was pursuing an MD-PhD at Columbia University, the HIV epidemic exploded to become a pandemic tremendously impacting both South Africa, as well as New York City. This motivated me to obtain a graduate degree in the study of HIV virology and immunology in the laboratory of Jeremy Luban. It was while studying HIV as a pathogen that I became interested in the potential application of lentiviruses for gene therapy. I completed Internal Medicine residency at the Brigham and Women's Hospital, followed by an Infectious Diseases fellowship at the Brigham and Massachusetts General Hospitals, after which I continued research into HIV immunology and vaccines in the laboratory of the late Norman Letvin. Subsequently, I transitioned to clinical development roles at Vertex Pharmaceuticals and later bluebird bio, developing drugs for HCV,

oncology and rare diseases including sickle cell disease and cerebral adrenoleukodystrophy, culminating in the first approval of a gene therapy for transfusion-dependent beta thalassemia. Most recently, I entered the venture world, working with the private investment arm of OrbiMed Advisors LLC to help bring novel gene and cell therapies to the clinic.

Tell us what the focus of your efforts at the present time is and what motivates you.

- Expanding the repertoire of life-shortening diseases—monogenic, as well as cancer, autoimmune, and neurologic—to which gene and cell therapy can be applied to achieve potentially curative outcomes
- Advancing the technology for delivery of gene therapy, to make gene therapy more accessible to populations in need globally by making the process of delivery safer and less expensive
- Evaluating novel platforms for the prophylaxis and treatment of infectious diseases, including SARS-CoV-2 and HIV

What is your vision for the future and how would you overcome any challenges?

- For most, if not all, rare genetic diseases, a curative gene therapy will either be already available, or readily developable with a smooth approval pathway, for patients in all countries and from all walks of life; it will be affordable; and it will not require the use of toxic conditioning regimens
- For most cancers, chronic infectious diseases, including HIV, and autoimmune diseases, a synthetic immune response comprised of one or more different cell types, with multiple different genetic modifications, that can be generated from off-the-shelf fully allogeneic, and durably effective cells
- Panels of broadly neutralizing antibodies against the host of studied pathogens with pandemic potential (e.g. coronaviruses, influenza, ebola, etc..) will be available encoded in an injectable and durable DNA formulation, ready to be scaled up rapidly and deployed as passive immunization/ prophylaxis should a future epidemic emerge
- To achieve these goals will require the combined efforts of private investment, large pharmaceutical R & D, government support and academic innovation

If there is one thing that would make a difference to your efforts, what would it be?

More hours in the day (or fewer hours needed for sleep).

What is a fun fact about yourself that you would like to share?

In a past life, I imagine I was a mediocre ninja, as in this life I am a mediocre martial artist (Krav Maga), and mediocre rock-climber.

Whitepapers

Interested in learning more? Read our latest whitepapers on the Caring Cross website. Click below to access them.

- [Global Access to Human Gene Therapy: Lessons Learned from HIV](#)
 - [Centralized vs Decentralized Manufacturing of Personalized Cell Therapies: How to Implement Local Manufacturing of CAR T-Cells](#)
 - [Regulatory Considerations for Decentralized Manufacturing of Personalized Cell Therapies: A Path Forward for Commercialization of Decentralized Manufacturing of CAR-T Cell Therapies](#)
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Upcoming Events

We feature a Technology Education seminar series on the first Friday of every month.

Currently we are presenting a multi-part series on how to manufacture CAR-T cells and other gene therapy products in a place-of-care setting. These presentations will

feature a step-by-step series, with direct hands-on demonstrations, of how place-of care manufacturing can be accomplished.

Place-of-Care Manufacturing of CAR-T Cells – Practical Application (Part 2)

 [Register for the event](#)

Date: Sep 2, 2022

Time: 3:00pm EST / 12:00pm PT

Location: Zoom (link provided upon registration)

This event will last approximately 30-40 minutes and will consist of a presentation and Q&A session following.

On the third Friday of every month, we feature an international expert in cell and gene therapy. This September, please register to hear Lindsay Androski, JD, MBA, CFA. Lindsay directs the non-profit Roivant Social Ventures and is a leading voice in making advanced medicines affordable through technological and regulatory innovation. Don't miss her presentation:

Environmental, Social and Governance (ESG) goals for addressing disease on a global scale

 [Register for the event](#)

Date: Sep 16, 2022

Time: 3:00pm EST / 12:00pm PT

Location: Zoom (link provided upon registration)

This event will last approximately 30-40 minutes and will consist of a presentation and Q&A session following.

All our events are on Fridays at 3pm EST and require registration to access the live webinar. A recorded replay will be available to Caring Cross Community members only (**Membership is free**).

Recent Events

If you missed these recent events, click on the links below to view them.

- ➔ [CAR-T cells for rhabdomyosarcoma: lessons for solid tumor immunotherapy](#)
- ➔ [Place-of-Care Manufacturing of CAR-T Cells – Practical Application \(Part 1\)](#)
- ➔ [Local manufacture of anti CD19 CAR T cells to treat non-Hodgkin's lymphoma](#)
- ➔ [Place-of-care manufacturing of CAR-T cells, a practical guide /Part 1: Introduction and Overview featuring Jane Reese-Koc, MBA, Cell Therapy Operations Director, and Physician Dr. Marcos de Lima](#)
- ➔ [Surface modification of gene transfer vectors to facilitate entry into cellular targets of gene therapy](#)

If you are not a member, you can [become a member](#) (it's free!) and view all our past events.

Caring Cross Community

Please follow the link below to be enrolled for an in-depth seminar series designed to educate our community about cell and gene therapy. This series was designed by patient advocates, with a general audience in mind. Don't miss this unique opportunity to hear from international leaders in the field, designed just for you.

the DARE Community Cell and Gene Therapy webinar series

- ➔ **Register:** <https://bit.ly/3kapavg>

We are creating a membership community to connect healthcare professionals,

scientists and engineers, community advocates and business leaders that are on a mission to develop new advanced medicinal cures - and help make them affordable to all who need them.

Join us to collaborate in a group, learn from seminars and training, and gain access to job opportunities or internships.

[→ Become a member](#)

What else would you like to hear about in this newsletter?

Reply to let us know.

Thank you for being here, look out for another update every other month!

- Caring Cross

P.S. Are you following along with us on social media? Be the first to know about our progress and share in the conversation!

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