

THE CURE CHRONICLES

July 2023 ISSUE

Enabling affordable and accessible advanced medicines

Greetings, and welcome to our latest edition, featuring Dr. Deus Bazira in our Community Spotlight. We are excited to see advances in cell and gene therapy continue across many disease fronts and remain more motivated to advance technologies and approaches that decrease their cost. We are also sobered by a new publication that attempts to quantify the true impact of sickle cell disease. To quote the IHME (Institute for Health Metrics and Evaluation, University of Washington) headline, "Sickle Cell Disease is 11 Times More Deadly Than Previously Recorded."

We will present each in the News section below.

News

Here is the latest on what we are excited about:

➔ **\$2.9 Million gene therapy for a form of severe hemophilia was recently approved by the FDA.** Once again, a gene therapy, using an AAV5 vector, is one of the most expensive drugs in the world. Here is a link to this story from ABC news to an article by Mathew Perrone, AP Health Writer:

<https://abcnews.go.com/Health/wireStory/gene-therapy-severe-hemophilia-approved-fda-100508758>

And a nice summary from Paige Twenter, for Becker's Hospital Review:

BioMarin Pharmaceutical attained the first FDA approval for a gene therapy indicated to treat severe hemophilia A. Roctavian (valoctocogene roxaparvovec-

rvox) was approved June 29 to reduce bleeding in adults patients with the blood disorder, according to a news release from the San Rafael, Calif.-based drugmaker.

Every year, hemophilia A affects 1 in 5,000 male births, according to the CDC. Because of the rarity of the disorder, the approval was based on a study of about 130 patients. In the trial, the drug reduced annualized bleeding rate in hemophilia A patients, on average, by 52 percent.

Although the full New England Journal of Medicine article is paywalled, signing up for a free account grants access to a very nice video summary of the trial, outcomes that led to approval, and the questions that remain about the durability of the treatment, and immunogenicity of the gene vector:

<https://www.nejm.org/doi/10.1056/NEJMdo006918/full/>

→ We also would like to highlight a new article that describes more fully and quantitatively than previous publications the true impact of sickle cell disease.

A summary of the article's main points from the IHME website:

<https://www.healthdata.org/news-release/sickle-cell-disease-11-times-more-deadly-previously-recorded>

8M people suffer from the hereditary disease which disproportionately impacts children, adolescents, and young adults.

- **A new analysis** provides a more complete picture of sickle cell disease mortality burden by combining disease prevalence data in different age groups and trends in overall survival when factoring in resulting secondary conditions.
- When looking across all deaths, **sickle cell disease is a leading cause of mortality in children <5 years** as well as in youth 5–14 years and adults 15–49 years.
- **Half a million babies were born with sickle cell disease in 2021**, and 79% of these infants were in sub-Saharan Africa.
- The largest increases in total deaths due to sickle cell disease since 2000 were in South Asia and sub-Saharan Africa, with the **rise driven by population growth**.
- Data gaps and high burden in regions with historically elevated rates of sickle cell disease highlight the **urgent need for universal newborn screening systems** and increased access to sickle cell disease treatment.

A link to the open access journal article in Lancet Hematology is linked below:

[https://www.thelancet.com/journals/lanhae/article/PIIS2352-3026\(23\)00118-7/fulltext](https://www.thelancet.com/journals/lanhae/article/PIIS2352-3026(23)00118-7/fulltext)

These two milestones summarize perfectly why we do what we do here at Caring Cross. The technologies for cell and gene therapy are advancing rapidly. And this is revolutionary stuff – it would have been categorized as science fiction 20 years ago. But the mechanism that has developed to now deliver these therapies is optimized to produce very high priced products. We believe these new technologies, and how they are applied, should be far lower in price, while being studied, delivered, and made affordable to the populations that need them most. Please join us in developing partnerships and pathways to do just that. At Caring Cross, we recently entered into a research agreement with Drs. Naoya Uchida and John Tisdale of the NHLBI, NIH, who have developed a next generation lentiviral gene vector to treat sickle cell disease. We are working with their team, and our research partners at UC-Davis, led by Drs. Mehrdad Abedi and Jan Nolta to find ways to create new pathways to treat sickle cell disease patients. Our bottleneck is resources. Working with state and federal funding agencies restricts the pace and scale of our work to “fundable units.” Our non-profit is ready to partner with individuals or organizations who want to see the pace of our advances accelerate. We would be grateful to you, or any contacts you may have, to help end the suffering endured by those with sickle cell disease by accelerating these programs.

Community Leader Spotlight

Deus Bazira, DrPH, MPH, MBA

Inaugural Director Georgetown University Global Health Institute, Director Center for Global Health Practice and Impact at Georgetown University Medical Center and Associate Professor of Medicine



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

I trained as a pharmacist and later branched into business management, health economics, health policy and public health at the graduate level. I started my career in my home country Uganda, later moved into academia at University of Capetown in South Africa. I moved to the USA in the early 2000s to join a global initiative to scale-up HIV care and treatment in sub-Saharan Africa and the Caribbean. I later joined University of Maryland Baltimore to develop and expand the global health work there and this culminated in the establishment of a Center for International Health and Education, and I was privileged to be appointed its inaugural director. In 2019, I joined Georgetown University and co-founded the Center for Global Health Practice and Impact which I now direct. The mission of the center is “to sustainably improve health outcomes through generation and translation of scientific evidence into policy and practice”. The Center’s work is anchored in implementation science,

science of improvement, integrated delivery, behavioral and data science approaches to optimize outcomes related to management of infectious diseases, non-communicable diseases, maternal neonatal & child health plus health security. Earlier this year, the President of Georgetown appointed me to serve as the Inaugural Director of the Georgetown University Global Health Institute on top of my role as the Director of the Center. The Institute seeks to advance scalable and sustainable solutions to improve health outcomes worldwide and enhance resilience of health systems to respond effectively to global health threats. A common feature about my past and present positions is that I am always tapped to build new institutions. I get drawn to starting things from scratch. I see opportunities where others may see obstacles.

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

My focus today is no different from when I started my journey as a public health practitioner and health systems strengthening enthusiast. The level and magnitude of my work is what has changed. My motivation stems from the desire to address complex public health challenges that can be solved through uptake of existing scientific evidence, technological and therapeutic breakthroughs. The ever-widening gap between evidence, knowledge and practice is what drew me to implementation science – my current focus. At the start of my professional path, it pained me to see preventable deaths and diseases that could have been avoided if only access to essential medicines and basic health promotion practices was universal. Thirty years later, this problem persists and it is not just about lack of access to medicines alone, it affects all medical technologies, public health best practices, and research evidence. To answer your question: I am still motivated by the desire to narrow the gap between knowledge and practice; between innovation breakthroughs and uptake, and; by health disparities that get widened by the day.

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

I will spend the next decade focusing my work and energies to enhance interdisciplinary approaches and effective collaborations to solve existing and emerging global health challenges. I will do this through building effective partnerships; leveraging and channeling resources into building resilient health systems, and; developing sustainable, structured and replicable processes to catalyze innovations in integrated health delivery.

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

If the global health community could invest more in implementing proven solutions instead of spending more resources in problem identification/description. In other words, more action, and less talk.

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

I speak cow language. As a kid I grew up on a farm and took care of cows a lot and over time like all cattle people, I learned to sing songs for cows that aligned with cow moods and state of mind and played the "Endingidi" (Ugandan bowed string music instrument) – and I perfected that. I could communicate with cows to convey directives and to calm them down.

Whitepapers

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

- [Distributive Manufacturing of CD19 CAR-T Cells Using CliniMACS Prodigy: Feasibility and Real-World Experience from India](#)
 - [A place-of-care approach to CAR-T cell manufacturing](#)
 - [Global Access to Human Gene Therapy: Lessons Learned from HIV](#)
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Upcoming Events

Caring Cross presents a Technology Education seminar series on the first Friday of every month. In 2023, we will present a new series created by cell and gene industry expert Dr. Kevin Curran.

Exploring Cell Therapies, Focus on CAR-T

[!\[\]\(feabb98897b440bc8695a03336a6e2df_img.jpg\) **Register for the event**](#)

Date: **Jul 07, 2023**

Time: **3:00pm EST / 12:00pm PST**

Location: **Zoom** (link provided upon registration)

On the third Friday of every month, we feature an international expert in cell and gene therapy. This September please register to hear gene therapy expert Dr. Semih Tareen present:

Approaches and Considerations for in vivo Gene Delivery

[!\[\]\(3cb60d42b10e53f9522bb0b392c1c4cd_img.jpg\) **Register for the event**](#)

Date: **Sep 15, 2023**

Time: **3:00pm EST / 12:00pm PST**

Location: **Zoom** (link provided upon registration)

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

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

Recent Events

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

- [Clinical and Commercial Challenges with AAV Gene Therapies](#)
- [Engineering fusion proteins to enhance adoptive cell therapy and catalyze endogenous antitumor immunity](#)
- [AAV Gene Therapy Manufacturing](#)
- [Toward Molecular Cures for The First Molecular Disease, Cell and Gene Therapies for Sickle Cell Disease](#)
- [Focus on AAV Gene Therapy](#)

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

Caring Cross Community

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](#)
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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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