

THE CURE CHRONICLES

February 2023 ISSUE

Enabling affordable and accessible advanced medicines

Welcome to our latest issue of the Cure Chronicles. The big three news items for this issue are: 1) The launch of Vector BioMed, the first spin-out company from Caring Cross that will accelerate our mission and reach, 2) Exciting developments at the CROI meeting, 3) The abandonment of sickle cell disease therapy development programs by a number of companies for finance-driven, rather than data-driven decisions. And in our community spotlight we are pleased to feature, Dr. Cissy Kityo, Executive Director of the Joint Clinical Research Center (JCRC), in Kampala, Uganda.

We were happy to present some of our latest research at the CROI meeting in Seattle, WA this month. Our poster is available there and, on our web-site. Enthusiasm in the field continues for what is termed “HIV Cure Research.” Advances in defining the activity of broadly neutralizing antibodies, immune subsets such as NK cells, T cells, and engineered T cells – such CCR5 knock-out and anti-HIV CAR-T cells that control disease in anti-retroviral therapy interruption trials, was presented. The field continues to better define what makes for a true cure, to celebrate the five individuals who have been cured of their infection, and to better understand molecular interventions that may lock the integrated provirus in place (thus preventing re-expression). On the pharmacologic side, longer acting anti-retrovirals that may allow for dosing once every six months are being developed and tested, hoping to bolster current efforts at stemming the tide of HIV infection. Combined with PREP, there are many tools at hand that are looking for broader and deeper implementation by medical and public health officials.

We were also fortunate to reflect on 20 years of the PEPFAR program, in an overview presented by Dr. Anthony Fauci. That there were protesters outside the convention center when Dr. Fauci presented reminds us all that public information, patient advocacy, and pushing back against false narratives remains a core

component of cure research. As we develop curative cell and gene therapies, we must anticipate (rather than respond to) the potential for false narratives to develop. A session at CROI was presented on the topic of “pre-bunking” rather than debunking bad information circulated on social media platforms.

In addition to the excitement that a conference like CROI generates, the field received sobering news when 4 companies discontinued development of products for sickle cell disease. As **Dr. Jennifer Adair’s** abstracts below, the problem is that money was specifically raised by Wall Street based on the perceived need and treatment gap in sickle cell disease. For companies to drop these programs, or pivot to a “potentially” more profitable product pipeline, represents a use, or rather misuse (to put it mildly), of a patient population that has been ignored for far too long. The Global Gene Therapy Initiative (GGTI), is working with national and international societies and agencies to formulate a response. A few of the news links to this issue are featured below, here are some important points that Dr. Adair has summarized from GGTI discussions:

-At the American Society for Hematology (ASH) meeting in December, Vertex Pharmaceuticals and Crispr Therapeutics presented their clinical trial data which was compelling and will be submitting for approval to FDA. This treatment using CRISPR gene editing is in addition to Bluebird Bio’s treatment using lentiviral vector which is also pursuing FDA approval. Both approaches are ex vivo gene therapies. Other companies with research & development pipelines still in the works for SCD include Beam Therapeutics (base editing approach), Editas Medicine (also CRISPR gene editing approach), and Intellia, the company whose partnership with Novartis to develop an in vivo gene therapy to treat sickle cell disease ended this week, will still pursue another treatment approach independently.

-Because of this competitive landscape, the companies who announced this week all did so to acknowledge the success of Bluebird and Vertex/CRISPR and invest their money in other initiatives. Some of those (Novartis and Intellia) will continue to invest in sickle cell, just not the approaches described. Others like Graphite Bio and Sangamo will put their money elsewhere.

-The facilities and infrastructure needed to meet Phase 3 clinical trial criteria as a company are a massive investment for bringing these therapies to the regulatory approval finish line. The competition is fierce and if an approach which is further behind in development costs more to make than those of Vertex/CRISPR or Bluebird, then the financial benefit to continue is limiting.

What we can emphasize is:

- We need multiple approaches to ensure access.*
- None of the ex vivo approaches has been evaluated in low-resource settings.*
- Having only two options, both of which have not been tested in low resource settings, is devastating to the community.*
- There may be an opportunity to transfer technologies which are being discontinued for competitive landscape reasons (without compelling data to suggest they won't work) with emphasis on access in low-resource settings. This could be the time to start those conversations. In the past, this has been done for cancer drugs by government agencies. For example, when AOI Pharmaceuticals discontinued O6-benzylguanine in the early 2000's, NCI and the Cancer Therapy Evaluation Program (CTEP) took custody of the supply and storage to provide free access for researchers and clinical trials.*
- Another option would be for other commercial entities or investors to license these technologies. However, this will depend on what happens to the IP associated with these announcements, which at present is unclear.*

Caring Cross was founded as a non-profit, because we realized that the just development of cell and gene therapy for diseases like HIV and sickle cell disease will require it. A large pharma, high-cost model is not feasible to deliver cures to those who need them most. Caring Cross engages in both research and implementation activities in order to further the development of affordable workflows for cell and gene therapy. To do so, we spin out companies and work with partners who share this vision. All companies created by Caring Cross are "B corps," that is companies that have a public benefit in their charter. Our first company, Vector BioMed, will operate independently as a CDMO to serve the market for lentiviral gene vectors, and in the future expand to AAV and other gene vectors. Caring Cross has a dedicated percentage of the Vector BioMed production capacity, and a preferred low-cost pricing for Caring Cross sponsored projects. Thus, we have full assurance that we can develop our current clinical programs with the clinic-ready gene vector products that will be required. Follow the links below to read more about this very exciting new development.

News

Here is the latest on what we are excited about:

A) Links to articles about the formation of Vector BioMed:

[Boro Dropulić and Rimas Orentas, Caring Cross \(investmentreports.co\)](#)

→ Contract Pharma

[Caring Cross Spins Out Biomanufacturing Company Vector BioMed · BioBuzz](#)

→ BioPharma Dive

[A startup launches with plans to open up a gene and cell therapy bottleneck | BioPharma Dive](#)

BioPharma Reporter

[New US CDMO launched to address viral vector supply bottleneck \(biopharma-reporter.com\)](#)

→ Endpoints News

[Vector BioMed launches with \\$15M to produce backlogged CAR-T component – Endpoints News \(endpts.com\)](#)

Fierce Pharma (Story has been corrected)

[Vector BioMed debuts as viral vector CDMO with \\$15M funding \(fiercepharma.com\)](#)

Washington Business Journal

[Gaithersburg biotech Vector BioMed launches with \\$15M in new funding - Washington Business Journal \(bizjournals.com\)](#)

→ MedCity News

[Vector BioMed Archives - MedCity News](#)

B) Links to stories about cessation of sickle cell disease activities:

<https://endpts.com/sickle-cell-rd-hit-by-three-culls-in-one-day-at-novartis-intellia-sangamo-and-graphite-bio/>

<https://www.biopharmadive.com/news/intellia-graphite-sangamo-sickle-cell-gene-therapy-discontinue/643400/>

(overview stories, Novartis/Intellia, Sangamo, Graphite Bio)

<https://www.investorsobserver.com/news/qm-pr/8255218450027450>

(Graphite Bio story)

https://sicklecellanemianews.com/news/clinical-hold-placed-sickle-cell-disease-treatment-ftx-6058/?utm_source=SIC&utm_campaign=bbeb3f1986-SIC_ENL_3.0_US&utm_medium=email&utm_term=0_b01e3fbae8-bbeb3f1986-74539466

(Fulcrum Therapeutics)

Community Leader Spotlight



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

I'm a medical doctor and Public Health specialist with over 30 years' experience in clinical care and health research in HIV and related infections since 1992 when I joined the Joint Clinical Research Centre (JCRC) after graduating from Makerere College of Health Sciences (MaCHS). I became one of the pioneers to prescribe Antiretroviral Therapy (ART) for HIV treatment in sub-Saharan Africa - that same year I joined the Centre and thereafter was at the forefront of scaling up HIV treatment in a cost recovery network model which became one of the two case studies (the second was in Haiti) that informed PEPFAR that ART was feasible in LMICs. JCRC subsequently became the first recipient of a PEPFAR grant in the world to scale up ART access in the region.

Following further training at Johns Hopkins School of Public Health and the University of Amsterdam for my Masters and PhD, I served in positions of Research Coordinator; Deputy Director, Research and Clinical Services; Deputy Executive Director, before becoming the Executive Director in 2017 and Director for the JCRC College of American Pathologists (CAP) certified laboratories. I have conducted and/or coordinated over 80 research projects and care program grants for HIV and other health conditions. I have been Principal Investigator, Co-PI or investigator for operational, basic science, clinical, epidemiological and field trials of HIV treatment and associated infections including TB, as well as intervention studies

aimed at preventing HIV transmission and preparation for HIV vaccines. I'm currently the JCRC site PI for the JCRC Clinical Research Site (CRS) of the US NIH AIDS Clinical Trials Group (ACTG) network running for 17 years now at the Centre. Many of these pioneering research projects have informed policy and clinical practice in Uganda and other developing countries.

I played a crucial role in the development of the JCRC state of the art clinical and research laboratories equipped to perform a variety of tests and research studies including: molecular biology, HIV drug resistance (HIVDR), Tuberculosis, HIV vaccine trials, pharmacokinetic studies, clinical, and epidemiological research among others. I serve as an executive member on various local and international scientific committees in HIV research, prevention and treatment and have been providing mentorship to both local and international scientists who come to Uganda.

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

I'm keen to be involved in gene therapy as the new exciting frontier in medical research with the potential to cure HIV and other diseases like Sickle Cell disease that are highly prevalent in Uganda and sub-Saharan Africa. From 2020, I co-founded with Prof Jennifer Adair the alliance of experts in gene therapy to support bridge the existing gaps between developed and developing countries. The Global Gene Therapy Initiative (GGTI) was subsequently formed to tackle the barriers to LMIC inclusion in gene therapy development and is working with various stakeholders to simultaneously address identified key areas that are requisite to successful implementation of Gene Therapy in developing countries. I serve on the Advisory Board of Caring Cross.

The proof of concept that five HIV patients in the world have been cured from HIV through bone marrow transplant (4) and gene therapy (1) provides a lot of hope that the same principles but using more feasible approaches like gene therapy could provide a CURE for the 1.5m Ugandans and approx 25m people living with HIV in sub-Saharan Africa. But this region has to date been excluded from research and access to these treatment modalities. I would like to see a fundamental change in this paradigm.

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

The vision is to contribute to universal access especially in LMICs to scientific advances and treatments that have potential to revolutionize management of highly prevalent diseases in these regions. Accessibility will include having simple, affordable and sustainable products for these diseases. The challenges to this vision include the necessary capacity and funding to make this a reality.

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

The right partnerships with similar goals to support the existing gaps in capacity building and funding the translational research in regions that have been left behind.

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

My motto goes “ The only way to achieve the impossible is to believe it is possible”, so I work hard and seriously to make this true while I also laugh hard in equal measure !

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.

Focus on AAV Gene Therapy

[→ Register for the event](#)

Date: **April 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 March please register to hear Carol Houts, Chief Strategy Office for Germfree, Inc., present:

Cleanroom Requirements for Cell Therapy Manufacturing: Exploring the Distributed Point-of-Care Model

[→ Register for the event](#)

Join us for this webinar on the latest innovation in cell therapy manufacturing – the distributed point-of-care (POC) manufacturing model. In this session, we will explore the benefits and challenges of implementing a distributed POC model for cell therapy, which involves manufacturing the product in a decentralized manner, closer to the point of care.

Date: **March 17, 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.

- [Introduction to Cell and Gene Therapy](#)
- [HIV Cure Research: Scaling Up Participant-Centered Approaches, Strategic Socio-Behavioral Sciences and Ethics Research](#)
- [Learn and apply: Harnessing features of effective antiviral T cell responses to enhance adoptive T cell therapies for HIV](#)
- [Place-of-care manufacturing of CAR-T cells, a practical guide: an interview featuring Jane Reese-Koc, MBA, and Dr. Marcos de Lima](#)
- [Accelerating Progress towards an HIV Cure for Africa](#)
- [Place-of-Care Manufacturing of CAR-T Cells – Practical Application \(Part 4\)](#)

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

Caring Cross Community

The DARE Community Cell and Gene Therapy webinar series ended with a bang, with Dr. Boro Dropulic presenting the final webinar. All previous webinars are available on YouTube, and you can find them listed on the DARE web-site:

<https://www.daretofindacure.org/community-events>

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.

[!\[\]\(c507f772dba2b921f86777f01218e570_img.jpg\) **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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