April 3, 2020

Dear Friends of the ARM Foundation:

The COVID-19 pandemic is a global crisis that has fundamentally altered our lives and brings into dramatic relief the need for a better understanding of science, nature and the investment we must make in education to become as informed as possible about how we should respond as a community to meet this challenge. Given that the ARM Foundation is an independent charity with a mission to serve as a trusted education and information resource on the contributions cell and gene medicine can make to patients and caregivers, we are compelled to broaden the mandate to include public health and the response to the COVID-19 pandemic.

The ARM Foundation staff and partners are undertaking an inventory of resources in the cell and gene medicine community that can help patients and families exposed to COVID-19 better understand the underlying pathology of the virus and how advancements in cell and gene medicine can contribute to its diagnosis and treatment. Ultimately, the global community will require a vaccine that can significantly reduce COVID-19’s threat to public health.

Please find here some of the contributions cell and gene medicine are making towards finding treatments, diagnostic platforms and vaccines for COVID-19:

**Cell and Gene Medicine Technologies Used to Fight COVID-19 Pandemic:**

Note: This is not meant to be a compilation of all known efforts and contributions relating to each of these technology platforms. We will be adding to these lists (posted on the ARM Foundation website: www.thearmfoundation.org) as we become aware of additional information or programs. Please also visit the ARM website (www.alliancem.org) for additional information about how the cell and gene therapy community is responding to the COVID-19 pandemic.

- **mRNA Vaccine Platforms:** Natural and synthetic mRNA can instruct cells to express an antigen that will solicit an immune response to the COVID-19 virus.
  1. Sanofi Pasteur-Translate Bio
  2. Moderna-NIAID Clinical Trial
  3. CureVac-CEPI
  4. Pfizer-BioNtech-Fosun

- **DNA Based Vaccine Platforms:** Using a direct intra-tissue DNA injection, treated tissue will produce and secrete viral like antigens to prime the innate immune system.
  1. Cobra Biologics-Karolinska
  2. Ology Bio-Inovo Pharma-DOD

- **Mesenchymal Stem Cells:** MSC transplantation or secreted vesicles have shown in some studies to dramatically mitigate the burden and duration of Acute Respiratory Disease Syndrome (ARDS) thus providing an ability to treat some of the sickest patients currently requiring ventilators.
  1. Celltex
  2. Athersys
• **CRISPR-Cas Gene Editing Technologies:** Teams are working on RNA guides using CRISPR-Cas that recognize two signatures of COVID-19 viral RNA, which can be leveraged for diagnostic and potentially eventual therapeutic purposes

  1. [Virus targeted CRISPr Therapies](#)
  2. [CRISPr-Cas Diagnostic approached](#)
  3. [Doudna-UC Berkely-IGI](#)

• **Single Cycle Adenovirus Vector Vaccines:** Several major medical centers in the U.S. and Europe are working on a single cycle adenovirus vaccines that are not replicating and therefore cannot cause further infection in the treated individual. The adenovirus vaccine expresses an antigen similar to those on the surface of the coronavirus thus enabling the immune system to recognize the virus if the patient is infected.

  1. [CanSino Ph1 Clinical Trial](#)
  2. [Johnson & Johnson](#)
  3. [University of Oxford](#)

• **NK, T, and modified immune Cells:** Companies are beginning clinical trials to test NK and other immune cells (a form of white blood cells) as a treatment for the Covid-caused pneumonia where these cells could attack and neutralize the infected cells and mitigate inflammation.

  1. [Celularity](#)
  2. [Korean Green Cross](#)
  3. [Baylor-AlloVir](#)
  4. [Macrophage Inflammation Treatment](#)

Additional Information on traditional pharmaceutical vaccine efforts can be found on the WHO website [here](#) and on the Clinicaltrials.gov website [here](#).

**Questions About Impact on Clinical Trials:**

The Foundation has also been fielding inquiries about the impact of the COVID-19 on clinical trials involving cell and gene medicine, especially in the rare disease and oncology communities. Each trial is affected to varying degrees by its size, scope, impact on supplies and manufacturing schedules, availability of facilities and in many cases additional risks posed to patients/trial participants who are immune compromised. Please find here links to the most up-to-date information on the impact of the COVID-19 pandemic on cell and gene therapy clinical programs.

  1. [Global Genes](#)
  2. [American Society for Transplantation and Cellular Therapy](#)
  3. [FDA Guidance](#)
  4. [European Medicines Agency](#)

**Concerns/Questions About Treatment Claims for Unproven Therapies**

We are also aware of recent claims that stem cells can be used to treat individuals infected with COVID-19. Although stem cells currently are used to treat some blood, bone, skin and eye diseases, some immune
system disorders, and can be used to resupply the blood system after cancer therapy, there currently are no approved stem cell therapies for the prevention or treatment of COVID-19. The Foundation is coordinating efforts with ARM, ISCT, and ISSCR to provide current information regarding the use of unproven therapies, in particular those offered by some “stem cell clinics” or organizations operating outside of established regulatory guidelines.

As stated above, there are a number of companies investigating the potential for stem cells to treat COVID-19 and Acute Respiratory Distress Syndrome resulting from the virus. Please follow these links to learn more about these efforts.

1. International Society for Cell and Gene Therapy
2. Cell and Gene Insight Blog - ISCT
3. The Niche: Knoepfler Lab Stem Cell Blog
4. International Society for Stem Cell Research
5. FDA Information Regarding Unapproved Stem Cell Treatments

Other Informational Resources:

Please find here links to informational resources about the contributions of cell and gene medicine to fighting the COVID-19 epidemic.

1. Alliance for Regenerative Medicine
2. Canadian Centre for Regenerative Medicine
3. California Institute for Regenerative Medicine

Lastly, the ARM Foundation in fulfillment of its educational mission is committed to helping patients, caregivers, and the general public gain access to the most timely and relevant information on the role of cell and gene medicine in responding to the COVID-19 epidemic. If you have specific questions you would like to address to the Foundation and our partners, please email info@thearmfoundation.org with the subject line “CGTx Covid-19 Inquiry” and we will respond to you within 24 hours.

Our collective courage, faith, resourcefulness and commitment to accelerating the development of medicines that will help provide enduring solutions to public health threats such as COVID-19 will guide us through this crisis. We remain committed to making sure that patients, caregivers, and the general public have access to the most trusted, reliable, information available.

Warmest regards,

Morrie Ruffin, Executive Director

Stewart Parker, Chair