



Gene Correction For Cell-based Medicines

Bridging the Silos Between  
**Patients | Providers | General Public**  
Supporting the Journey of Curative Medicine

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# Outline: Bridging the Gaps Between Rare Disease Stakeholders and Curative Cell and Gene Therapies



- ▶ **Connecting with the patient community**
- ▶ **Creating a patient-centric approach**
- ▶ **Supporting unbiased educational initiatives**
- ▶ **Thoughtful utilization of study tools and vendor partners**

# Introduction

- Kamau Therapeutics is a clinical-stage, next-generation gene correction company harnessing high efficiency targeted gene integration to develop a new class of therapies with the aim to cure a wide range of serious and life-threatening diseases
- Over 7 years of experience working in rare-disease space – specifically sickle cell disease (SCD)
- Currently enrolling ph1/2 study – autologous gene editing for SCD
- Previous experience running global phase 3 SCD studies



Memorial Sloan Kettering  
Cancer Center™

# Connecting with the Patient Community

Gaining insight into the perspectives and experiences of patients, their families, and other stakeholders across all phases of clinical trials

- Building a network with Patient Advocacy Groups (PAOs) and Community Based Organizations (CBOs)
  - Align with a patient advocacy though leader to support thoughtful outreach and engagement (internal resource, CRO resource, etc.)
  - Understand and learn more about the community
  - Focus attention on the various regions where your study is conducted as the community may differ from region to region
  - Show up to support and give back to the patient community
  - Open door of communication with the patient community
  - Don't bite off more than you can chew to ensure your outreach remains thoughtful and consistent
  - Internal initiatives to spread engagement and awareness within your company (share the love!)

# Connecting with the Patient Community

Gaining insight into the perspectives and experiences of patients, their families, and other stakeholders across all phases of clinical trials

- Leveraging patient input through focus groups to ensure that recruitment strategies and clinical trial protocols reflect the needs, concerns, and preferences of the patient community
  - Patient lead focus groups to review patient facing materials
  - Collect feedback from the community – What’s working and what isn’t? How can we show up and/or do better?
  - Through teaching CBOs and PAOs about your study and mechanism of action, they can in-turn teach the community and spread awareness about your clinical trial
  - Leverage PAO and CBO clinical trial resources and databases to support enrollment and engagement
  - Real-time feedback engaging all stakeholders on your clinical protocol (patients, caregivers, patient advocates)

# Creating a Patient-Centric Approach

Building a model that prioritizes patient needs and experiences, from initial research (bench) to clinical application (bedside)

- Dissecting the clinical protocol with all stakeholders in mind
  - Sponsor needs
    - Clinical endpoints
    - Biostatistical considerations
    - Regulatory considerations
    - Bar set by past clinical protocols
  - Providers
    - Site capabilities and limitations
    - Resourcing
    - Standard of care
  - Patients and caregivers
    - Physical, mental and emotional limitations and considerations
    - Health care disparities
    - Keeping patient burden to a minimum

# Creating a Patient-Centric Approach

Building a model that prioritizes patient needs and experiences, from initial research (bench) to clinical application (bedside)

- Incorporating the lived experience designed to support enrollment and protocol compliance
  - Leveraging patient focus groups at the start of protocol development
  - Maintain moment and feedback loop with PAOs and CBOs through life of a clinical study
  - Keep a pulse on the community and therapeutic landscape as a whole; keeping in mind all stakeholders
  - Patient Navigator support to best understand the needs of the patients on study in real time
  - Incorporate consistent patient tools and resources from the start of a clinical study to ensure regulatory compliance

# Supporting Unbiased Educational Initiatives

Championing efforts to inform and educate stakeholders about rare diseases, therapies, and the clinical trial process without bias

- Ongoing community engagement
  - Ongoing collaboration with CBOs and PAOs with the support of a Patient Advocate lead
  - Leveraging community educational talks, resources and social media
  - Giving back to the community (fundraising, walks, monetary sponsorship, etc.)
  - Conference attendance
  - Social media
  - Podcasts
- Internal initiatives to provide education and community connection
  - Organize company events to support patient engagement and education
  - Invite patients to share their stories with your company
  - Encourage company events tied to fundraising or giving back to your CBO or PAO network

# Utilizing Study Tools and Partners Thoughtfully

Selecting and using the right tools and vendors to effectively support patient enrollment, engagement, regulatory compliance, data collection, and long-term follow up

- Identifying tools to support the needs of patients as well as your specific clinical protocol
  - Patient navigator resources
  - Patient advocacy support
  - Support groups and mental health
  - Travel support and compensation
  - Patient stipends
  - Caregiver support (travel, lodging, stipend)
  - Fertility preservation
  - PROs
  - Wearables

# Utilizing Study Tools and Partners Thoughtfully

Selecting and using the right tools and vendors to effectively support patient enrollment, engagement, regulatory compliance, data collection, and long-term follow up

- Identifying tools to support the needs of patients as well as your specific clinical protocol
  - Geographical location of sites
  - Size of study/number of patients to be screened and enrolled
  - Study population and demographics
  - Specific needs of your patient population
  - Financial considerations
  - Leveraging tools already in place through clinical sites, PAOs, CBOs, and community

**THANK YOU!**

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