Gene Therapy Guide

Objectives
- Understand the risks associated with gene & cell therapy
- Describe the targets, techniques and cell types used in HIV gene & cell therapy
- Describe how gene & cell therapies fit into HIV cure efforts

Materials
- PowerPoint Deck
- Find Your Match Activity
- Case Study
- Pre/Post Test Assessments

Estimated Time
PowerPoint Deck- 45-60 minutes
Find Your Match Activity- 30-45 minutes
Case Study- 20-30 minutes
Pre/Post Test Assessments- 5-10 minutes

Planning for a Workshop
Trainers should plan an agenda for the session or workshop. The format of the module can be used as a guide and structure. When designing an agenda trainers should take into consideration the three factors below.

Knowledge level of trainees: Any previous knowledge or experience with HIV cure research or information should be the primary consideration. If the pre-assessment exams are given out prior to the workshop they can provide some insight into a group’s literacy level.

Sector of trainees: The entire curriculum is geared to community, particularly areas where research is being planned or conducted. Trainers may want to adjust the knowledge or content slightly based on the audience.

Total length of the workshop: Approximate times are listed for each component of the module. Trainers should allow for additional time for activities such as: introductions and establishing group norms, coffee/water stretch breaks and work-shop wrap up. Trainers may also want to consider including additional modules, such as Basic HIV and Cure module.
PowerPoint Animations
Familiarize yourself with the animations that occur throughout the PowerPoint. Ensure that you are comfortable with all of the animations and their purpose- found in the slide notes- prior to giving the presentation.

Slide 3: 5 clicks in total:
- “HIV+ Acute Myeloid Leukemia Patient”
- “Identification of HLA-identical, CCR5 32 homozygous bone marrow donor”
- “Chem- and Radiotherapy conditioning”
- “Allogeneic stem cell transplant”
- “6 years later: remains cured”

Slide 8: 2 clicks in total
- The first click to advance from slide 7 to 8 will trigger the picture to float in
- A therapeutic HIV protection gene will appear and move into the blood cells

Slide 9: 5 clicks in total:
- The “X” appears at the receptor gene target and after each click with move to the next potential target until it reaches the budding stage.

Slide 11: Five clicks total:
- “Antigen binding component” and “Expressed on outside of cell; This can be part...”
- “Usually binds HIV envelope on infected cell”
- “HLA independent”
- “Signaling component” and “Sends signal into cell”
- “Directs the cell to kill HIV infected target” and the red “Binds viral protein”

Slide 12: 17 clicks in total:
- The picture of the human body appears and slides to the left.
- “Mobilization” appears
- “Leukapheresis or Bone Marrow Harvest” and arrow
- Blood cell cluster appears
- “Virus-mediated transfer...” and vector appear
- Vector moves into the blood cluster
- Clicks 7-8 fade in and out of microscope photo of chromosome
- Clicks 9-10 fade in and out of microscope photo of chromosome
- Clicks 11-12 fade in and out of microscope photo of chromosome
- “Reinfusion” and another curved arrow appear
- GOAL: Gene therapy appear
- Cancer
- Genetic disease
- Infectious disease
Slide 14: “HIV target gene if CCR5” and arrow appear after click

Slide 15: 6 click in total:
  - Vector appears from left side
  - Arrow and DNA strand with yellow coding appear
  - A “pacman” type structure appears inside the cell
  - The DNA with orange shading is broken in half
  - A blue arrow appears and the orange shaded DNA is repaired as a yellow shaded DNA
  - The title of the slide appears

Slide 16: 10 click in total:
  - Patient appears
  - Arrow, “Collection HSCs” and cells appear
  - Small arrow appears on right side
  - “Vector mediated gene...” appear
  - “Nucleases for CCR5...” appear
  - “Nucleases to eliminate...” appear
  - Arrow, larger collection of cells, “Expanding gene-edited...” appear
  - Another arrow
  - Patient and “Development of novel...” appear
  - Arrow, photo of blood cells and “Generation HIV protected...”