

# 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- 30-45minutes

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 will 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 click 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...”

**Slide 27:** “Development of novel conditioning...” and “Expansion of gene-edited...” appear