

Molecular Therapy

Letter to the Editor

Implementation of a gene therapy education initiative by the ASGCT and Muhimbili University of Health and Allied Sciences

There has been rapid growth in gene therapy development with an expanding list of approved clinical products. Several therapies are particularly relevant to patients in low- and middle-income countries. Moreover, investing in research and manufacturing presents an opportunity for economic development. To increase awareness of gene therapy, the American Society of Gene and Cell Therapy partnered with the Muhimbili University of Health and Allied Sciences, Tanzania, to create a certificate-bearing course. The goal was to provide faculty teaching in graduate and medical schools with the tools needed to add gene therapy to the university curriculum. The first virtual course was held in October of 2022, and 45 individuals from 9 countries in Africa completed the training. The content was new to approximately two-thirds of participants, with the remaining third indicating that the course increased their knowledge base. The program was well received and will be adapted for other under-resourced regions.

INTRODUCTION

The first approved clinical trial of gene transfer was conducted over 30 years ago.¹ Since that time, there has been continued growth in technology and indications. Currently, there are an estimated 2,000 gene therapies under development,² and over 60 approved products are predicted to enter the market by 2030.³

Pharmaceutical companies have received criticisms as many approved gene therapies are the most expensive treatments with some priced at over 2 million dollars (US).^{4–7} This has threatened access to needed therapies, particularly for patients in low-resource regions. Moreover, many of

the diseases prevalent in low- and middle-income countries (LMICs) are actually high-priority gene therapy targets (sickle cell anemia [SCA], hemophilia, cancer, and human immunodeficiency virus [HIV] infection).⁸ As a result, some LMICs are opting for in-country product development and manufacturing infrastructure to improve access and foster economic development.^{2,9} Such investments require workforce development that includes greater knowledge of gene therapy technology and clinical indications. Unfortunately, studies assessing genetic literacy are limited. Looking at individuals with secondary education, Chapman et al. surveyed 5,404 individuals from 78 countries and found genetic knowledge to be poor in most individuals.¹⁰ Other studies also note limited genetic literacy.^{11,12} Acceptance of gene therapy is more likely in families with familial disorders,¹³ but it may be limited in populations with an underlying mistrust of the medical system^{14,15} and when they involve *in utero* gene editing.¹⁶ Therefore, improving literacy in genetics could help foster workforce development and impact government policies, public awareness, and acceptance of gene therapy within a healthcare system.

To increase awareness of gene therapy, the Global Outreach Committee of the American Society of Gene and Cell Therapy (ASGCT) partnered with the Muhimbili University of Health and Allied Sciences (MUHAS) to create the Introduction to Gene Therapy for Educators (IGTE) course. This 8-session certificate course was designed to provide an educational opportunity for teaching faculty at LMIC. The goal was to equip course participants with the skills needed to develop a gene therapy curriculum for their home graduate or medical school. This manuscript highlights the development, implementation, and lessons learned from the first course held in the fall of October 2022.

COURSE CONCEPTUALIZATION AND DEVELOPMENT

ASGCT is a not-for-profit professional organization of over 7,000 members with a mission “to advance knowledge, awareness, and education leading to the discovery and

clinical application of genetic and cellular therapies to alleviate human disease.” Members of the ASGCT Global Outreach Committee suggested IGTE as a means of meeting the educational goals of the Society. The MUHAS started as the Dar es Salaam medical school in 1963 under the University of Dar es Salaam, and by 2007, it had become a full-fledged university. The objectives of the University are the advancement of knowledge, diffusion, and extension of technology and learning, the provision of higher education and research, and, so far as is consistent with those objectives, the nurturing of the intellectual, aesthetic, social, and moral growth of the students at the University. The Department of Biochemistry and Molecular Biology in the Campus College of Medicine, School of Biomedical Sciences, within MUHAS hosted the IGTE course. Modern biochemistry has grown to encompass a wide range of areas that overlap molecular biology, cell biology, and genetics. The department has 15 full-time faculty and 3 supporting staff of whom, apart from teaching biochemistry and molecular biology and genetics courses to both undergraduate and postgraduate students, serve as faculty advisors.

The course description is provided in [Figure 1](#). Admission criteria included employment as a full-time academic faculty at an accredited university or medical school and a desire to add gene therapy to the curriculum. Acceptance in the course required applicants to provide a *curriculum vitae* and answer a series of screening questions regarding current teaching activities. MUHAS was responsible for course admission, administration, and awarding the course certificate.

Course designers received suggestions from the ASGCT Global Outreach Committee and MUHAS faculty on basic concepts important in developing a gene therapy curriculum, including suggestions for instructors and content experts to deliver the content. A goal was to incorporate critical concepts into 8 90-min sessions (a 60-min didactic lecture followed by a 30-min discussion). Other design decisions include delivering the sessions in English over 4 weeks using the Zoom meeting platform. The 60-min

Course purpose:

To equip the participants with the necessary knowledge, attitude, and skills on the advances in clinical gene therapy to facilitate the inclusion of gene therapy education into university and medical school curricula.

Course duration:

The course will be held on eight days given over 4 weeks with 2 sessions given per week. The course will be virtual with each session lasting 1.5 hours with 1 hour dedicated to didactic content and 0.5 hours to discussion.

Course objectives:

Participants completing the course will learn:

1. Methods used in genetic modification of human tissues
2. Major disease targets for clinical gene therapies
3. Ethical and regulatory challenges to gene therapies
4. Limitations and opportunities for equitable access to gene therapy

Course learning outcomes:

Knowledge: Explain the methods used in clinical gene therapy,

Summarize the status of gene therapy clinical trials

Skills: Create educational materials to educate trainees on the current status of clinical gene therapy

Attitudes: Critique the ethical and regulatory issues regarding genetic modification of human tissues

Instructional methods: Lecture, Discussion, Self-directed learning, Small group work, Collaborative learning, Case study

Figure 1. Course description and objectives

lectures were live to allow the participants to engage with the instructors. Each course had one instructor and a second content expert whose role was to answer questions as they appeared in the chat. The combined lecture and discussion were recorded and available to attendees after the live session. Supplemental reading material was made available on Dropbox (www.dropbox.com).

Objectives and outcomes were established for each of the 8 planned sessions (Table S1 supplemental data). For content development, the course organizers engaged 29 ASGCT members to contribute potential content based on their expertise. For each of the 8 sessions, a lead developer was appointed to draft lecture material and present

the final content during the course. ASGCT staff reviewed all slides to ensure the lectures were the same length, the slides had a uniform style, and relevant citations were included. Specific diseases were selected to provide examples of different methods for gene correction (e.g., non-viral and viral vectors, CRISPR, and other editing tools), as well as topics relevant to the audience (leukemia, SCA, HIV, hemophilia, Parkinson disease, and blindness). Lectures were developed in an iterative process with the course organizers to ensure continuity between sessions. Feedback was provided to emphasize concepts that had broad applicability to gene therapy, as opposed to specific facts or figures that are likely to change over time. Concepts were reinforced over subsequent

sessions. For example, the genetics of SCA was addressed in session 1, with gene therapy approaches to SCA discussed in session 2, and current SCA clinical trials in session 3. Later sessions touched on ethical and advocacy issues, as well as regulatory aspects relevant to SCA and other genetic disorders.

A flier with course topics, learning objectives, and information about the application process was circulated by MUHAS and the Sickle Pan-African Research Consortium (SPARCo), including all SPARCo contacts within Africa. The advertisement was also shared through the MUHAS website and social networks (Instagram, Twitter, WhatsApp, and Facebook). The course was also advertised through ASGCT's Twitter account, and an e-mail was sent to all ASGCT contacts who reside in Africa. The enrollment goal was limited to 60 participants. Surveys used in assessing course content, delivery, and participant demographics were anonymized, and use of collected data for this manuscript was approved by the MUHAS Senate Research and Publication Committee.

COURSE IMPLEMENTATION

The course was held in October of 2022 and was free to eligible participants. Of 52 applicants, 49 met the acceptance criteria, and 45 from 9 countries attended the course and received certificates (Figure 2A). All participants were from countries that met the World Bank definition of an LMIC. In terms of program metrics, the average module length was 89 min and attendees remained on the Zoom session an average of 78 min. Speaker participation was 100% despite their varied geographic locations (North and South America, Europe, Africa, and India). One session was rescheduled shortly after the course started due to an unanticipated conflict with the speaker's schedule.

There was an average of 30.5 participants per live session (range 23–37), with decreasing participation in the live sessions as the course progressed (Table S1, supplemental data). All sessions were recorded and made available to participants afterward. Of the 7 participants who attended fewer than 4 of the live lectures, they requested the recording

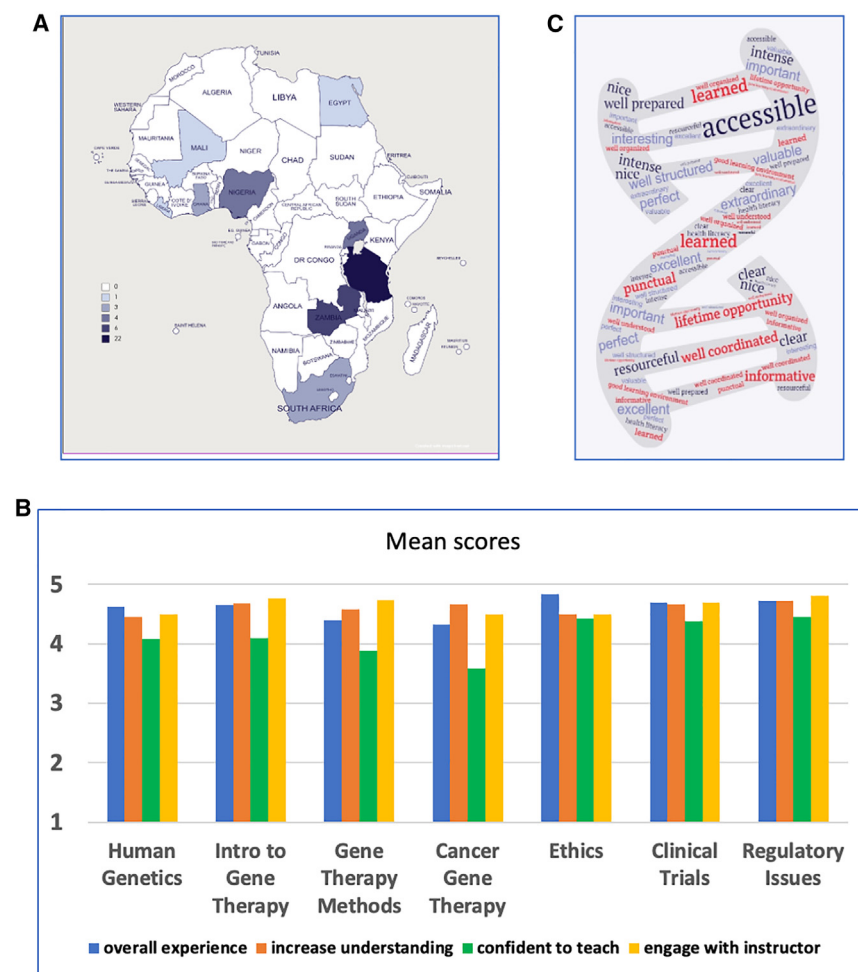


Figure 2. Participant Demographic and Course Assessment

(A) Participants by country. A total of 45 participants from 9 countries graduated from the course. (B) Rating parameters and feedback on the course. Attendees were asked the following questions for each of the modules. (1) How would you rate your overall experience in today's module? (1 being not positive and 5 being very positive). (2) To what extent did the module increase your understanding of the topic? (1 being no increase and 5 being a significant increase). (3) How confident are you that you can educate others about what you have learned today? (1 being not confident and 5 being very confident). (4) How do you feel you were able to engage with the instructor? (1 being difficult to engage and 5 being easy to engage). Session 8 is not shown as fewer than 10 attendees rated the session. The number of participants providing feedback for sessions 1–7 were 35%, 66%, 46%, 39%, 44%, 67%, and 27%, respectively. (C) Participants shared their feedback about the course using the following words to describe the experience.

of the session immediately following their absence. At the conclusion of each lesson and at the end of the entire course, attendees had an opportunity to rate the overall experience, whether the session increased their understanding of the topic, their confidence in being able to teach the material, and their ability to engage with the instructor. In general, all the sessions were well received; see Figure 2B.

Participants were asked to identify the sessions that they learned the most from. The sessions that focused on gene therapy basic science issues (sessions 2, 3, and 4) were rated the highest. The sessions on ethics, regulatory issues, and advocacy (sessions 5, 7, and 8) received the lowest scores with attendees stating relevance to their area of work as the reason. Interestingly, attendees rated the ethics and regulatory sessions high-

est when asked about confidence to teach the material (Figure 2B). When soliciting topics that should be included in future courses, several participants identified training on educating lay audiences and advocating for the public understanding of genetic diseases. It should be noted that participants were a mix of scientific and medical professionals, which may account for differences in rating (Bachelor 10, Master 9, PhD 7, MD-PhD 3, and 19 with MBBS/MMed/MD). Moreover, course assessments were voluntary, and overall participation was 42%. Additional efforts to promote greater involvement in course assessments should be incorporated in future course offerings.

Participants had the ability to provide overall feedback at the end of the course. A word map was generated from end-of-course responses and is shown in Figure 2C. In general, the responses were highly positive, and no major negative themes emerged. Approximately two-thirds indicated the content was new information, while the remaining third indicated the information increased their knowledge base. Eighty-seven percent indicated the session was about the right length (90 min), and the ability to understand the presenters' spoken English had a mean rating of 4.73 (1 being too difficult and 5 being easy to understand). When asked about alternative formats for the course, 60% would prefer a hybrid virtual/in-person course. The multiple presenter format was viewed favorably by 97% of participants, which allowed them to engage with several subject matter experts rather than one instructor for the duration of the course. When asked "How prepared do you feel to apply what you have learned to your current work?" the mean rating was 4.0 (1 being not at all prepared and 5 being very prepared), and when asked, "How well did you understand the concepts covered in the course?" the mean rating was 4.1 (1 being too difficult and 5 being easy to understand).

CONCLUSIONS AND LESSONS LEARNED

The IGTE course received overwhelmingly positive feedback from the participants. Key to this response was the partnership between MUHAS and ASGCT, the effective

course administration by MUHAS faculty in the Biochemistry Department, and the willingness of volunteer gene therapy experts to develop the course content. This course was very relevant to MUHAS faculty as gene and cell therapy is part of the course content for both undergraduate and postgraduate programs at their institution. The overall format appeared to work well for participants, although feedback suggests the time devoted to ethical, regulatory, and advocacy content could be reduced. Continuing to offer this program for Africa appears worthwhile, and adapting it to other under-resourced regions appears appropriate. This course will be repeated, in partnership with MUHAS, with a stronger focus on the basics of gene therapy. Attendance and other requirements for certification will now be stated in the application materials. Given the feedback, each module will also include a short quiz, so participants can test their knowledge, and instructors will incorporate a review at the start of the following module. Based on the lessons learned from this course, ASGCT has planned several additional initiatives to facilitate the training and education of learners and young career professionals in other LMICs. Additionally, ASGCT will continue to develop partnerships in other regions of the world with the aim of offering an in-person course.

SUPPLEMENTAL INFORMATION

Supplemental information can be found online at <https://doi.org/10.1016/j.ymthe.2023.07.019>.

ACKNOWLEDGMENTS

We also wish to thank the following who contributed to the initial development of course content: Julie Makani, Nori Kasahara, Jesse Gitaka, Serene Forte, Paola Grandi, Daniela Bischof, Sunil Raikar, Meisam Naeimi Kararoudi, Stephan Hart, Thomas Wechsler, Punam Malik, Megan Waldrop, Alessandro Aiuti, Ulrike Reiss, January McKee, Federico Mingozzi, Kaye Spratt, Laura Hameed, Rachel Salzman, and Michelle Berg. Martin Bonamino is supported by CNPq – National Council for Scientific and Technological Development (grant # 313106/2020-7 and 311761/2018-6), FAPERJ - Foundation for Research

Support of the State of Rio de Janeiro (grant # CNE-E–26/202.645/2019).

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All authors participated in the conceptualization, design, implementation, and writing the manuscript and in reviewing and editing the manuscript.

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<https://doi.org/10.1016/j.ymthe.2023.07.019>

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REFERENCES

- Rosenberg, S.A., Aebersold, P., Cornetta, K., Kasid, A., Morgan, R.A., Moen, R., Karson, E.M., Lotze, M.T., Yang, J.C., Topalian, S.L., et al. (1990). Gene transfer into humans-immunotherapy of patients with advanced melanoma, using tumor infiltrating lymphocytes modified by retroviral gene transduction. *N. Engl. J. Med.* 323, 570–578.
- Forum, W.E. (2022). Accelerating Global Access to Gene Therapies: Case Studies from Low- and Middle-Income Countries. *World Econ. Forum.* <https://www.weforum.org/whitepapers/accelerating-global-access-to-gene-therapies-case-studies-from-low-and-middle-income-countries/>.
- MIT NEWSDIGS Research Brief (2020). Updated projection of US durable cell and gene therapies product-indication approvals based on December 2019 development pipeline. *Mass. Inst. Technol. FoCUS.* <https://newdigs.mit.edu/sites/default/files/NEWDIGS-Research-Brief-2020F207v51-PipelineAnalysis.pdf>.
- Richardson, M., Rind, D., Beaudoin, F.L., Pearson, S.D., and Campbell, J.D. (2023). The Fair Price For One-Time Treatments; How Can We Overcome Existing Market Price Distortions? *HealthAffairs.* <https://doi.org/10.1377/forefront.20230117.20110613>.
- Senior, M. (2017). After Glybera's withdrawal, what's next for gene therapy? *Nat. Biotechnol.* 35, 491–492.
- Kolata, G. (2017). New gene-therapy treatments will carry whopping price tags. *In New York Times.*
- National Public Radio (2019). At \$2.1 Million, New Gene Therapy Is The Most Expensive Drug Ever. <https://www.npr.org/sections/health-shots/2019/05/24/725404168/at-2-125-million-new-gene-therapy-is-the-most-expensive-drug-ever>.
- Cornetta, K., Patel, K., Wanjiku, C.M., and Busakhala, N. (2018). Equitable Access to Gene Therapy: A Call to Action for the American Society of Gene and Cell Therapy. *Mol. Ther.* 26, 2715–2716.
- Cornetta, K., Bonamino, M., Mahlangu, J., Mingozzi, F., Rangarajan, S., and Rao, J. (2022). Gene therapy access: Global challenges, opportunities, and views from Brazil, South Africa, and India. *Mol. Ther.* 30, 2122–2129.
- Chapman, R., Likhanov, M., Selita, F., Zakharov, I., Smith-Woolley, E., and Kovas, Y. (2019). New literacy challenge for the twenty-first century: genetic knowledge is poor even among well educated. *J. Community Genet.* 10, 73–84.
- AlRasheed, M.M., AlAli, H., Alsuwaid, A.F., Khalaf, S., Ata, S.I., BinDhim, N.F., Bakheet, D., Khurshid, F., and Alhawassi, T.M. (2021). Gene Therapy Knowledge and Attitude Among Healthcare Professionals: A Cross-Sectional Study. *Front. Public Health* 9, 773175.
- Mboowa, G., and Sserwadda, I. (2019). Role of genomics literacy in reducing the burden of common genetic diseases in Africa. *Mol. Genet. Genomic Med.* 7, e00776.

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Letter to the Editor

13. Adams, D., Roche, L., and Heussler, H. (2020). Parent perceptions, beliefs, and fears around genetic treatments and cures for children with Angelman syndrome. *Am. J. Med. Genet. A.* *182*, 1716–1724.
14. King, W.D., Wyatt, G.E., Liu, H., Williams, J.K., DiNardo, A.D., and Mitsuyasu, R.T. (2010). Pilot assessment of HIV gene therapy-hematopoietic stem cell clinical trial acceptability among minority patients and their advisors. *J. Natl. Med. Assoc.* *102*, 1123–1128.
15. Dubé, K., Simoni, J., Louella, M., Sylla, L., Mohamed, Z.H., Patel, H., Luter, S., and Collier, A.C. (2019). Acceptability of Cell and Gene Therapy for Curing HIV Infection Among People Living with HIV in the Northwestern United States: A Qualitative Study. *AIDS Res. Hum. Retroviruses* *35*, 649–659.
16. Vazquez-Loarte, T.C., Lucas, T.L., Harris-Wai, J., and Bowen, D.J. (2020). Beliefs and Values About Gene Therapy and In-Utero Gene Editing in Patients with Hemophilia and Their Relatives. *Patient* *13*, 633–642.

Supplemental Information

Implementation of a gene therapy education

initiative by the ASGCT and Muhimbili

University of Health and Allied Sciences

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Supplemental Data. Table 1. Objectives and Expected Outcomes for Gene Therapy Training Sessions (n = number of participating trainees attending the live session)

Session	Objectives	Outcomes
Genetics of human disorders (n = 37)	1. Genetic and epigenetic regulation of cell growth and function 2. Genetic basis for monogenic diseases 3. Non-coding mutations and disease 4. Non-Mendelian inheritance	1. Recall the organization of the human genome 2. Explain gene regulation of cell function 3. Differentiate the different types of genetic changes that lead to disease 4. Design a lecture on gene therapy that incorporates the relevant genetic mechanisms of human disease
Gene therapy overview (n=35)	1. The history of human gene therapy 2. Gene delivery using viral and non-viral vectors 3. Gene editing approaches to genome modification 4. Vector selection based on target tissue and therapeutic goals	1. Summarize the different methods use to alter genetic sequences 2. Use online databases and medical literature to keep current on advances in gene therapy 3. Differentiate gene therapies ideally suited to ex vivo applications to those requiring in vivo administration 4. Design lectures on clinical gene therapy that include the most current advances in the field
Methods for correcting genetic diseases (n = 37)	1. Gene therapy for monogenic diseases 2. Use of gene transfer as drug delivery 3. Novel approaches to complex genetic disease 4. Risks associated with genetic modifications	1. Compare the advantages and disadvantages of gene transfer methods when treating different target tissues 2. Explain potential approaches to treating complex genetic diseases 3. Differentiate the risk and benefits of viral vectors and gene editing 4. Generate a lecture that provides an overview of gene therapy for monogenic and complex genetic diseases
Use of gene therapy in cancer treatments (n = 31)	1. Genetic approaches to altering cancer cell growth 2. Engineering autologous T cells to eliminate cancer cells 3. Genetically modified viruses for cancer therapy 4. Cancer vaccines	1. Recall the mechanism by which the immune system can mediate tumor killing 2. Explain the mechanism of action for various cancer gene therapies 3. Critique the current gene therapy approaches to cancer therapies 4. Design a lecture that provides an overview of cancer gene therapy

Ethical issues in genetic alterations (n = 27)	1. Terminology used in describing genetic therapy and genetic engineering 2. Informed consent issues in gene therapy 3. Unintended consequences of gene therapy 4. Ethical issues surrounding modification of somatic and germline tissues	1. Explain the basic principles of biomedical ethics 2. Differentiate the ethical issues associated with somatic and germline tissues 3. Contrast ethical issues as they impact societal and individual interests 4. Design educational material that conveys the ethical issues applicable to gene therapy
Current status of clinical gene therapy (n = 24)	1. Worldwide distribution of gene therapy clinical trials 2. Countries with approved gene therapy products 3. Predictions for future gene therapy products 4. Limitations in providing gene therapy to under-resources areas	1. Identify the high priority diseases in gene therapy development 2. Explain the status clinical trial development and approved products 3. Summarize the resources required to provide ex vivo and in vivo gene therapies 4. Generate educational materials on the worldwide status of gene therapies
Regulatory challenges to implementing gene therapies (n = 30)	1. Biosafety oversight common to gene therapy regulations 2. Regulatory oversight of drug products 3. Regulatory challenges specific to cell and gene therapy products 4. Country variability in oversight of cell and gene therapy	1. Identify biosafety risks based on the gene therapy delivery system 2. Compare the Phases of clinical trial as they relate to drug product development 3. Research the regulatory infrastructure present in the learner's home country 4. Create educational materials that summarize regulatory issues important in gene therapy
Patient advocacy groups and international partnerships in fostering access to novel therapies (n = 23)	1. Cost challenges in gene therapies 2. Models to promote advanced therapies in under-resourced areas 3. Patient advocacy role in influencing investigators, government agencies, and industry 4. International partnership models for improved access to care	1. Identify the factors that contribute to the high cost of gene therapies 2. Compare different approaches to promoting access to gene therapy clinical trials 3. Explain the role of advocacy groups and foundations promoting access to novel therapies 4. Create educational materials that capture the challenges of providing access to gene therapy in under-served areas and populations