American Society of Gene + Cell Therapy

ASGCT Draft response to call for contributions from the WHO Expert Advisory Committee on Developing Global Standards for Governance and Oversight of Human Genome Editing

Draft framework: https://www.who.int/docs/default-source/ethics/governance-framework-for-human-genome-editing-2ndonlineconsult.pdf
** Submission was via online text box, not a signed letter*

Name: [no answer]
Affiliation: American Society of Gene & Cell Therapy
Country of Residence: United States
Have you read the draft framework: yes

Please comment on the approach taken by the Committee in developing its DRAFT Governance Framework?

The American Society of Gene & Cell Therapy (ASGCT) applauds the Committee for addressing this important, challenging, and highly complex scientific, ethical, and societal issue. ASGCT is a nonprofit professional membership organization comprised of more than 4,400 scientists, physicians, and other professionals working in gene and cell therapy in settings such as universities, hospitals, and biotechnology companies.

The Society appreciates this opportunity to be supportive of the Committee efforts and to provide feedback throughout this process. We commend the Committee for actively seeking input from a variety of stakeholders, including institutions, organizations, communities, and peoples often underrepresented in international science policy processes. ASGCT would recommend including in the framework a list of the organizational stakeholders that participated in the process.

Box 3 in this draft framework, which breaks down the regulatory analysis conducted thus far by region, is beneficial. We recommend inclusion of additional details on the methodology for this analysis to clarify whether areas outside of Europe and the Americas have fewer collected documents because more of such documents do not exist or because of a need for further outreach in those areas to assess the status of positions. If the latter is the case, ASGCT recommends additional outreach to countries in underrepresented regions.

As stated in previous comments, the Society believes somatic cell gene editing should not be subjected to an undue burden of scrutiny that is not applied to other innovative therapies, and generally opposes additional accreditation, registration, or licensing requirements for somatic cell editing that exceed those applied to other therapeutic products in countries with frameworks in place for regulating gene therapy, including gene editing. Therefore, while safety monitoring requirements of somatic cell gene-edited products beyond approval is advisable at this time, we would view the details of post-market research requirements, as well as whether and how to enforce them, to be within the realm of regulators.

Please provide your opinions on the specific proposals relating to governance of human genome editing specific considerations for good governance in the DRAFT Governance Framework (Part 3)?

Item 19: This listing of the phases of gene editing research and types includes "enhancement." ASGCT would not consider enhancement to be a phase, but rather a potential intended purpose of gene editing. The Society does not view gene editing for non-therapeutic enhancement of body performance or features as an acceptable use of gene editing.

Item 21 & Part 3.1 (Special Challenge: Heritable Human Genome Editing): The Society is pleased the Committee highlights the crucial distinctions that must be made between gene editing in somatic and germ cells. Additionally, we appreciate the inclusion of definitional differences between germline genome editing (used only for basic research purposes) and heritable genome editing (used clinically to attempt to achieve reproduction). ASGCT strongly encourages the Committee to continue to emphasize its interim recommendation that "it would be irresponsible at this time for anyone to proceed with clinical applications of human germline genome editing." As an interim goal to the establishment of broad societal consensus on the future use of this technology, we support the continued issuance of clear international and national statements on the current status of the science.

Item 25: Given the paramount position of patient safety and wellbeing in the Committee's work, ASGCT applauds its recognition of the risks of illegitimate and unscrupulous gene editing treatments, and its commitment to including "measures to prohibit human genome editing travel or tourism and have disciplinary tools to deter unscrupulous behaviors." Proactive prevention efforts are critically important in deterring misuse akin to that currently occurring in clinics offering unapproved stem cell uses.

Item 27: ASGCT has concern over the statement, "It is certain that there will be significant differences in the policy directions taken by countries around the world regarding prohibition versus permission (usually within a regulatory regime) [of heritable human genome editing]. Good governance must anticipate this and plan for these variations." The Society does not view the permission of heritable human genome editing to be inevitable. We recommend the Committee propose an international oversight mechanism to prevent heritable germline gene editing, which is nearly universally opposed at this time, including by the Committee and WHO Director-General Tedros Adhanom Ghebreyesus.

The statement in this item does not acknowledge the possibility that heritable germline gene editing may never be deemed to be safe and/or ethical for testing in humans. ASGCT supports a strong ban on heritable germline gene editing unless and until the technical and ethical problems can be solved, broadly and deeply discussed, and societal consensus reached.

Part 3.4 (Special Challenges: Post-Natal Human Somatic Gene Editing): The Committee highlights valid potential benefits, risks, challenges, and opportunities of somatic gene editing. However, ASGCT views the risks of somatic cell gene editing as neither entirely unique nor unpredictable. We respectfully disagree that existing regulations are insufficient with respect to details for somatic gene editing trials. While many countries would benefit from the development of dedicated regulatory frameworks to address the unique aspects of all types of gene therapy, including gene editing, the Society does not view governance on a global scale of somatic cell gene editing as necessary or desirable. Rather, we encourage reliance upon

best practices from more advanced regulatory frameworks and greater convergence of regulatory requirements among individual countries for all types of gene therapy, including gene editing, to facilitate safe, efficient development.

Please comment on the tools, institutions, and processes for human genome editing governance in the DRAFT Governance Framework (Part 4)?

ASGCT appreciates the Committee's thorough summary of the tools, institutions, and processes that may contribute to human genome editing governance. The Committee's descriptive analysis is quite informative of the ways in which laws, judicial decisions, ministerial decrees, research funding, professional self-regulation, and research ethics guidelines may act and interact to regulate gene editing technologies in different contexts. The Society encourages the Committee in its further work to provide recommendations on the creation and/or use of international and/or national mechanisms of oversight of heritable gene editing.

Item 70. ASGCT appreciates mention of the contribution to oversight of scientific societies in their provision of a forum for professional self-reflection. ASGCT advocates for the responsible use of gene and cell-based technology and organizes events that address heritable gene editing and other issues related to responsible and ethical use.

Part 4.11 (Interest Groups and Public Influencers): ASGCT is pleased to see the evolution of this point since the Committee's previous draft, particularly that groups such as biohackers are excluded from the list of relevant influencers and stakeholders. The Society does recommend that patient advocacy organizations are acknowledged as a distinct group in the "interest groups" category. While ASGCT does agree that other interest groups may be referenced broadly, patients and their families are at the heart of this discussion and should be recognized as such.

Please provide your opinions on the scenarios in the DRAFT Governance Framework (Part 5), including whether we have missed any important details?

[Answer left blank.]

Please comment on the questions to be considered when developing governance measures (Annex)?

The Annex identifies questions a country should consider when developing national governance measures. The section on Heritable Human Genome Editing (for reproduction) includes a question on if pre-clinical and clinical research on heritable human genome editing will be
permitted under a country's governance framework. ASGCT's position is that clinical research on heritable human genome editing should not be permitted at this time and that therefore this question should not be included.

What would you want to see in a decision tree to assist those taking governance decisions? (We are currently consider creating a decision tree based on the questions to be considered when developing governance measures (Annex))?

[Answer left blank.]

Are there additional measures we could include to deter or avoid bad practice around applications of human genome editing (such as rogue clinics or other 'bad actors', inappropriate uses of the technology, etc.)?

[Plan to leave blank.]

What else do you want to tell us about good governance of human genome editing?

As the Committee notes in this framework, technology advancements will require governing bodies and other stakeholders to continually respond and adapt to new developments. ASGCT appreciates the Committee's deep engagement with these challenging issues and looks forward to the next draft of the governance framework.