Executive Summary of Implementation Workshop on
'WHO Considerations in Developing a Regulatory Framework for Human Cells and Tissues and for Advanced Therapy Medicinal Products'

24-26 September 2025
Brazzaville, Congo

The implementation workshop on WHO considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products was held in Brazzaville, Congo from 24 to 26 September 2025.

A total of 40 participants attended the workshop. These included 15 regulators representing national regulatory authorities (NRAs) from 14 countries in the African region, as well as 2 regulators from Egypt, the only country from the Eastern Mediterranean region. Furthermore, there were 8 regulators and experts from 5 WHO regions serving as facilitators and invited speakers, 7 representatives from manufacturers, and 7 WHO staff from headquarters (HQ) and the WHO Regional Office for Africa (AFRO), participating both in person and virtually. In addition, a representative from the African Union Development Agency-New Partnership for Africa's Development (AUDA-NEPAD) took part in the workshop.

Fig 1. Countries represented by regulators

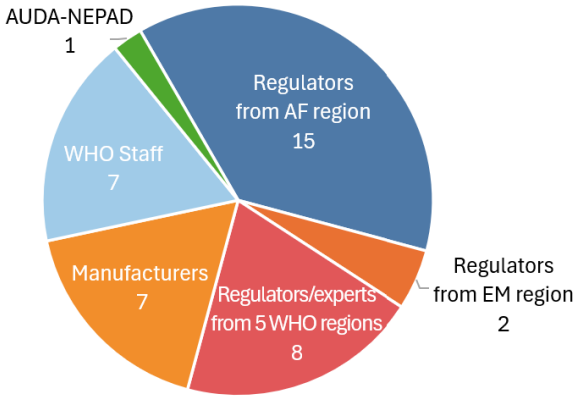

Fig 2. Composition of participants

This was the second implementation workshop, building on the outcomes of the first workshop held in Oman (2024)[1] and reflecting the recommendations from the 19th ICDRA meeting (2024)[2]. The objectives of the workshop were to:

[1] Executive Summary of WHO Implementation Workshop on 'WHO Considerations in Developing a Regulatory Framework for Human Cells and Tissues and for Advanced Therapy Medicinal Products'
[2] WHO Drug Information - Volume 38, No. 4

- Provide NRAs with key principles of the WHO considerations to facilitate their implementation into national practices.
- Share regulatory and development experiences, as well as country situations and plans for respective frameworks.
- Discuss opportunities for collaboration to establish or improve regulatory frameworks and to promote regulatory convergence.

Over the three-day workshop, the key principles outlined in the WHO document "Considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products (WHO TRS No. 1048, Annex 3, 2023)[3]" were discussed in ten open sessions, with particular focus on the African context. The detailed agenda of the workshop is provided at the end of this document.

The sessions began with an overview of WHO activities in biological standardization, followed by discussions on the risk-based approach for the regulation of CGTPs, including product classification and terminology. The regulatory status of CGTPs in different countries was shared and discussed by regulators, together with their experiences, challenges, and areas where WHO support is needed. Manufacturers provided perspectives on the current state of CGTP development, and discussions were also held on clinical trials of CGTPs. Further discussions focused on the implementation of the WHO considerations document, including plans to be developed by countries to establish and strengthen regulatory frameworks, the support needed from WHO, and approaches to build regulatory capacity.

Group activities on three case studies were conducted, covering products ranging from minimally manipulated tissues to advanced therapy medicinal products requiring comprehensive regulatory oversight. These exercises provided participants with an opportunity to apply risk-based regulatory approaches in practice.Through these discussions, participants emphasized the importance of clearly defining safety, manipulation, classification, and terminology. Challenges highlighted included difficulties in classifying products, the regulatory complexity of gene therapy products, and the need for training in regulatory evaluation, including GMP requirements. The limited number of clinical trials and marketing authorization applications for CGTPs in many African countries was noted as a barrier, as it restricts practical exposure and hands-on experience, suggesting that capacity constraints and the absence of guidance may limit development.

Table 1. Case studies on CGTPs for risk-based regulatory approach

	Products	Description of product classes	Risk considerations related to classification
Case 1	Irradiated, sterile human skin allograft	• Minimally manufactured tissue used for same function in recipient as in donor	• No manipulation or minimal manipulation • Same intended use in donor and recipient

[3] Considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products

Case 2	Autologous genetically modified hematopoietic stem cells for treatment of sickle cell anemia	• Biological products requiring full premarket authorization with a demonstration of safety and efficacy	• Substantial manipulation of the cellular starting material • Product-specific non-clinical and clinical data required to support the indication
Case 3	Allogeneic pancreatic islet cells and Mesenchymal stem cells (MSCs)	• Manufactured tissue used for same function in recipient as in donor	• More than minimal manufacturing or non-homologous use in the recipient

Manufacturers shared perspectives on pathways to improve access to CGTPs in low- and middle-income countries. They recommended measures such as optimizing or establishing GMP facilities, creating regional hubs, fostering international training partnerships, integrating CGTP-related curricula into universities, and developing indigenous supply chains. They also emphasized the importance of regulatory convergence across the region, the establishment of fast-track pathways, and the development of country-specific frameworks, including those for rare diseases.

Participants expressed strong support for the WHO considerations document and agreed on the need for capacity building, both at the level of national regulatory systems and among regulatory staff. Specific needs identified included training on CGTP evaluation, the establishment of dedicated teams for ATMPs, and mechanisms to ensure long-term follow-up of patients, particularly for gene therapy products. The value of reliance mechanisms, joint reviews, and regional cooperation was underlined as essential to support countries with limited resources.

Several cross-cutting issues were also raised. Medical tourism was identified as a global concern, with participants noting that insufficient regulation in some countries exposes patients to unsafe products while creating additional burdens on health systems when adverse events occur. The complexity of regulating products prepared in hospitals was also highlighted, particularly in settings where exemptions allow therapies to be provided without oversight or where responsibilities are divided among multiple authorities.

Participants requested that WHO continue to support the development and implementation of regulatory frameworks for CGTPs. Specific areas of support identified included:

- Capacity building for both regulatory systems and staff, highlighted as a priority by many countries;
- Provision of coordination, training, and other technical support;
- Assistance in developing or adapting local regulatory frameworks in line with the WHO considerations document;
- Development of comprehensive guidelines covering both pre-market and post-market regulation of CGTPs;
- Facilitation of cooperation among countries, including mechanisms for information and document sharing;
- Establishment of structured training programmes and specialized capacity building for regulatory staff;

- Development of guidance documents and practical toolkits tailored to the needs of the African region;
- Promotion of regional regulatory networks and reliance pathways to strengthen efficiency and patient access;
- Support for public–private partnerships to enhance GMP capacity and expand local manufacturing.

The workshop concluded with consensus on the need for sustained collaboration among NRAs, manufacturers, and WHO to ensure that regulatory frameworks for CGTPs are strengthened both in the African region and globally. Such efforts were considered essential to enable safe, effective, and timely patient access to these innovative therapies.

WHO Implementation Workshop:
WHO Considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products

24 – 26 September 2025, Brazzaville, Congo

AGENDA

Chair: I. Reischl
Rapporteur: G. Jotwani

Day 1: 24 September 2025, Wednesday

Session 1	**Welcome and introduction**	
09:00 – 10:00	Opening remarks and welcome speech	K. Nyarko, WHO AFRO I. Knezevic, WHO HQ B. Impouma, WHO AFRO
	Group photo	All participants
	Self-introduction	All participants
	Statement on DoI assessment	I. Knezevic, WHO HQ
	Housekeeping information	WHO AFRO
Session 2	**Background and objectives**	
10:00 – 10:20	Update on WHO biological standardization	I. Knezevic, WHO HQ
10:20 – 10:30	Objectives and expected outcomes of the workshop	E. Kim, WHO HQ
10:30 – 11:00	***Coffee Break***	
Session 3	**WHO Considerations on cell and gene therapy products (CGTPs)** *Considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products (Annex 3, WHO TRS 1048, 2023)*	
11:00 – 11:30	Risk-based regulatory approach, CGTP classification and terminology	I. Reischl, AGES-MEA[i]
11:30 – 12:30	Q&A and discussion	Chair & facilitators
12:30 – 13:30	***Lunch break***	

Session 4	**Case studies on CGTPs (1)**	
13:30 – 14:00	Brief explanation of case studies 1	I. Reischl, AGES-MEA
	Minimally manufactured tissue used for same function in recipient as in donor: Irradiated, sterile human skin allograft	
14:00 – 15:30	Group work *(Participants will be divided into groups to discuss.)*	All participants
15:30 – 16:00	***Coffee break***	
16:00 – 17:00	Reporting of the outcomes	Rapporteur(or leader) from each Group
17:00 – 17:50	Open discussion & summary	Chair
17:50 – 18:00	**Wrap-up of Day 1**	Chair

Day 2: 25 September 2025, Thursday

Session 5	**Current practices in CGTPs from regulatory perspectives**	
09:00 – 09:20	Feedback summary from countries	E. Kim, WHO HQ
09:20 – 10:20	Roundtable discussion by NRAs *Regulatory experience and challenges (e.g. establishment of regulatory framework, regulatory collaboration/convergence)*	NRAs & Chair
10:20 – 10:30	Q & A and summary	Chair
10:30 – 11:00	***Coffee Break***	
Session 6	**Current practices in CGTPs from industry/clinical perspectives**	
11:00 – 11:20	DCVMN perspectives on cell & gene therapy products manufacturing for developing countries	S. Kale, DCVMN[ii], Serum Institute of India
11:20 – 11:35	IFPMA's view on development of ATMPs and challenges	K. Ho, IFPMA[iii], Roche
11:35 – 11:50	Sickle cell disease and cell and gene therapy-based clinical trials	R. K. Bakyayita, Seattle Children's Research Institute
11:50 – 12:05	Ethics and regulatory challenges in clinical trials	H. Opolot, UNCST[iv]
12:05 – 12:30	Q&A and discussion	Chair

12:30 – 13:30	***Lunch break***	
Session 7	**Case studies on CGTPs (2)**	
13:30 – 14:00	Brief explanation of case study 2 *Biological products requiring full premarket authorization with a demonstration of safety and efficacy: Gene therapy products for treatment of sickle cell disease (SCD)*	M. Schüessler-Lenz
14:00 – 15:30	Group work *(Participants will be divided into groups to discuss.)*	All participants
15:30 – 16:00	***Coffee Break***	
16:00 – 17:00	Reporting of the outcomes	Rapporteur(or leader) from each Group
17:00 – 17:50	Open discussion & summary	Chair
17:50 – 18:00	**Wrap-up of Day 2**	Chair

Day 3: 26 September 2025, Friday

Session 8	**Case studies on CGTPs (3)**	
09:00 – 09:30	Brief explanation of case study 3 *Manufactured tissue used for same function in recipient as in donor : Allogeneic pancreatic islet cells and MSCs*	Y. Maruyama, PMDA[v]
09:30 – 10:40	Group work *(Participants will be divided into groups to discuss.)*	All participants
10:40 – 11:10	***Coffee Break***	
11:10 – 12:10	Reporting of the outcomes	Rapporteur(or leader) from each Group
12:10 – 12:30	Open discussion & summary	Chair
12:30 – 13:30	***Lunch break***	

Session 9	**Implementation of WHO considerations**	
13:30 – 14:30	Discussion: How to implement WHO considerations, *e.g. tools to facilitate, support needed from WHO*	All participants
Session 10	**Conclusions and way forward**	
14:30 – 15:00	Summary report of points raised during the workshop	Chair & Rapporteur
15:00 – 15:15	Conclusions and next steps	Chair & Rapporteur
15:15 – 15:30	Closing remark	
15:30	**Close of the open meeting**	
15:30 – 16:00	***Coffee Break***	
Session 11	**Closed session (regulators and participants without conflict of interest)**	
16:00 – 17:00	Feedback from countries: *e.g. implementation plan, global/regional collaborative activities*	All NRAs
17:00 – 17:20	Discussion	All NRAs
17:20 – 17:30	**Close of meeting**	**Chair & WHO**

[i] AGES-MEA: Austrian Medicines and Medical Devices Agency
[ii] DCVMN: Developing Countries Vaccine Manufacturers Network
[iii] IFPMA: The International Federation of Pharmaceutical Manufacturers & Associations
[iv] UNCST: Uganda National Council for Science and Technology
[v] PMDA: Pharmaceuticals and Medical Devices Agency