BRITISH PHARMACOPOEIA COMMISSION

Working Party ATMP: Advanced Therapy Medicinal Products 7th Meeting

SUMMARY MINUTES

A hybrid meeting of this Expert Advisory Group was held in person and via MS Teams on Monday 11 November 2024 commencing at 10:00 AM and continuing into the afternoon.

Present: Dr Jacqueline Barry, Chris Burns, Kimberly Gilmour, Leanne Di Rollo, Rickard Nordström, Josefina Nilsson, Caroline Weydert, Clare Blue, Rita Tendeiro Rego, Ramond Mendoza, Peter Emery-Billcliff, Francis Galaway, Christian van den Bos, Sabine Domning, Sarah Kempster, Edward Mee, Paul Getty, Leonard Pattenden, Alicja Fiedorowicz, Paolo Tozzi, Anna Nowocin, Keith McLuckie, Sushmita "Mimi" Roy, Jennifer Buckland

In attendance: Mr Kajetan Rakowski, Mr Ryan Smith, Ms Rachael Feltham, Dr Ian Rees, Mr Reece Dowling, Dr Janet Glassford, Dr Claire Kerridge.

Observing: Ms Jennifer Buckland

1. General Matters **ATMP(24)01**

The Chair reminded members that any Freedom of Information requests should be forwarded to the BP team. Members were also reminded of the confidential nature of the documents related to this work, and not to share anything marked as "Official Sensitive" outside of the group.

Members were asked to inform the BP team if their contact details had changed. A general list of Duties of members was attached to the meeting papers for reference.

A link to the 2023 annual report of the Human Medicine Regulations advisory bodies was included in the papers. The report was published in July 2024 and included the BP Commission Annual Report.

2. Progress Report **ATMP(24)02**

Two guidance documents were published this year following public consultations and comment responses from the group. There were approximately 300 comments from responders, which each of the subgroups considered before publication. The "Characterisation of Capsid Particle Population for rAAV Products" guidance was published on 12 January 2024. The "T Cell and NK Cell Characterisation Assays" guidance was published on 8 April 2024. There were over 1300 unique registered downloaders for the ATMP guidance so far. The group then heard about ongoing work that allowed the wider WP ATMP to comment and review documents prior to public consultation.

3. Replication competent virus assays draft guidance review **ATMP(24)03**

The Scientific Lead, for the replication competent virus assay guidance acknowledged the team's extensive work. Initial approaches were revisited and restructured to refine the

guidance, the content was simplified by grouping common guidance for AAV and lentiviruses to improve usability. Feedback was received and the next steps agreed.

4. PCR validation protocols and reports review ATMP(24)04

The PCR subgroup presented an update on their work to revise the 2021 PCR validation guidelines. Due to methodological differences, separate sets of protocols and reports were developed for AAV (adeno-associated virus) and LVV (lentiviral vectors). The goal was to align with the updated ICH Q2 (R2) guidance on analytical validation and industry standards. A draft of the updated guidance and annexes, including two protocols and two reports, were completed. The guidance document underwent several key updates, including terminology changes, updates and new sections. Validation attributes and workflows were updated to align with the new ICH Q2 (R2) guidance, streamlining certain parameters. WP ATMP members were asked to provide comment, prior to finalisation of the draft for public consultation.

5. Encapsidated DNA characterisation ATMP(24)05

The group heard a short update from the Scientific Lead of the encapsidated DNA characterisation subgroup. This update outlined the scope of the guidance, which was intended to be a part two to the characterisation of the capsid particle population guidance which was published earlier this year. It was agreed that the methods that would be focussed on were as follows:

- Next generation sequencing (Long read)
- Next generation sequencing (Short read)
- qPCR
- dPCR (including ddPCR)
- Alkaline denaturing gel
- Capillary electrophoresis

One expert suggested the addition of Cryo-TEM to this list and offered their support to the subgroup should they require it.

6. Capsid protein characterisation ATMP(24)06

The group heard a short update regarding the capsid protein characterisation subgroup. This update outlined the scope of the guidance, which was intended to be a part three to the characterisation of the capsid particle population guidance which was published earlier this year. It was agreed that the methods that would be focussed on were as follows:

- LC-Mass Spectrometry
- Liquid chromatography-UV/FLR
- AAA/LC
- Antibody-based techniques: ELISA

- Affinity-based techniques: SPR and interferometry
- SDS-PAGE
- Capillary Gel electrophoresis UV/FLR
- CEMS
- cIEF UV/FLR
- Bioinformatics tools
- Amino acid analysis

7. Future work programme workshop **ATMP(24)07**

A workshop was held assessing two potential future optics: CRISPR CAS9 off target effects and CMC Considerations for Cell-Based ATMP Cryopreserved Products.

8. Point of Care Manufacturing **Verbal update**

An overview of the background and progress of the modular manufacture and point of care amendments to HMR and clinical trials regulations was presented to the group The amendments aimed to accommodate new manufacturing methods, including portable on-demand units and other relocatable manufacturing sites, which could operate near hospitals or other locations. The group were informed of the current progress of the legislative changes and upcoming schedule. The group was given the opportunity to get involved with the guidance writing process at the appropriate time.