BRITISH PHARMACOPOEIA COMMISSION

Working Party ATMP: Advanced Therapy Medicinal Products 6th Meeting

SUMMARY MINUTES

A meeting of the working party was held at 13:00 via MS Teams teleconference on Thursday 7th December 2023.

Present: Jacqueline Barry (Chair), Edward Mee, Alicja Fiedorowicz, Leanne Di Rollo, John Campbell, Len Pattenden, James Norton, Caroline Weydert, Huimin Tao, Rita Tendeiro Rego, Clare Blue, Wei Wang, Victoria Vanhoutte, Paul Carter, Zara Hannoun, Isobel Searing, Lyn Healy, McIntosh Jenny, Mark Lowdell, Kimberly Gilmour, Tishwant Kanwarjit, Keith McLuckie, Chaminda Salgado, Sabine Domning, and Paul Getty

In attendance: Ryan Smith, Kajetan Rakowski

Apologies from: Peter Emery-Billcliff, Lily Li, Stephen Vinter

1. General Matters ATMP(23)01

Jacqueline Barry 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 2022 annual report of the Human Medicine Regulations advisory bodies was included in the papers. The report was published in July 2023 and included the BP Commission Annual Report.

2. Progress Report ATMP(23)02

Since the fifth WP ATMP meeting the BP team had established subgroups for two topics: Replication-competent virus (RCV) assays and Validation protocols and reports for digital and quantitative polymerase chain reaction (PCR) for vector copy number. Both subgroups had their kick-off meetings, assigned scientific leads as well as identified knowledge gaps within the groups. Profile for Experts documents were created and used to identify new external experts, who were subsequently endorsed by the BP Commission.

Kajetan Rakowski provided a non-technical update from four of the subgroups of WP ATMP.

2.1 Replication competent virus assays – members were informed that the subgroup aims to produce a guidance document that would be applicable to those who intend to conduct replication competent virus assays in house as well as those who intend to outsource the testing to contract development and manufacturing organisations. This would be done by providing the considerations needed for each and allowing the user to make their decision based on their product and available facilities.

2.2 dPCR and qPCR validation protocols and reports for VCN – members were made aware that this subgroup aimed to develop documents for dPCR and qPCR validation protocol

and report templates for LV/AAV VCN that would be annexed to the published VCN guidance. The LV and AAV were separated into different documents due to their differences in validation and controls.

2.3 Characterisation of the capsid particle population for rAAV products – the guidance document had been posted for public consultation between November 2022 and January 2023. The subgroup considered all 274 comments, and the finalised version has been passed over to TSO for web publishing as BP website-only content behind a paywall and as a freely downloadable PDF.

The subgroup members were thanked and praised for their contributions to this guidance.

2.4 T cell and NK cell characterisation assays – The public consultation for T cell and NK cell characterisation assays ran from April 2023 to May 2023. The subgroup had considered all 157 comments received and the finalised guidance document has been passed over to TSO for web publishing. The group were made aware that the guidance would be made available in Q1 2024.

The subgroup members were thanked and praised for their contributions to this guidance.

3. Replication competent virus assays draft guidance review ATMP(23)03

Dr Carter gave a technical update to the group about the progress made within the replication competent virus assay guidance subgroup. The update began by describing how the document usage was envisaged to be used by different stakeholders, with this in mind he explained the decision taken to split the document up by virus type sections. This structure would specifically include ATMP virus types such as LV, AAV, and Adeno.

4. PCR validation protocols and reports review ATMP(23)04

Ms Fiedorowicz gave a technical update to the group about the progress made within the PCR validation protocols and reports subgroup. The objectives of the subgroup were outlined.

The group was informed of a definition change for VCN to AAV genomic titre, which Dr Blue, the scientific lead for the vector copy number subgroup agreed with this proposal along with others from the group. The definition would remain the same for LV.

5. Horizon scanning 2023 ATMP(23)05

Mr Smith presented a summary of the cell and gene therapy standardisation-related work carried out by organisations other than MHRA. This included work by the Standards Coordinating Body for Regenerative Medicine, European Pharmacopoeia, and ISO Gene Delivery Package.

6. New subgroup proposal ATMP(23)06

The group were made aware of plans to explore encapsidated DNA characterisation and primary protein sequence modifications. These two topics would be closely related to the Characterisation of the capsid particle population for rAAV products subgroup and would likely form a Part 2 and Part 3 to the guidance. Volunteers were sought to be involved with a scoping and profile for expert building meeting.

7. Closing remarks

Members were made aware that action points and minutes will be circulated by email.

The Chair thanked everyone and informed the group that the BP team would be in touch via email correspondence to arrange a 2024 meeting and gain endorsement on proposals on future work established during this meeting.

Clare Blue and Lily Li were thanked for their contributions to a recent BioPharma Asia Webinar in November 2023. The contents of the webinar included vector copy number and characterisation of the capsid particle population in rAAV products. The webinar was recorded and made available for on demand watching on the BioPharma Asia website.