FACILITATING THE AUTHORISATION OF PREPARATION PROCESS FOR BLOOD, TISSUES AND CELLS

INTERIM NEWSLETTER 2019

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OVERVIEW OF GAPP INTERIM NEWSLETTER

We dedicate this issue to GAPP interim meeting held in Rome on October 29th and 30th 2019 because the dissemination team believe, as Dr Giancarlo Maria Liumbruno, Director general of the National Blood Centre (CNS) said at the opening, *"the outcomes of GAPP action are of paramount importance, and this GAPP action is really looking into the future of facilitating innovation in BTC for patient benefit"*. The aim of the meeting was to present the updates of the project to the whole consortium, European Commission officers and External Advisory Board members. Forty-seven people among collaborating stakeholders, project beneficiaries and 1 EU Commission Officer attended the meeting.

BACKGROUND

GAPP is the acronym (Facilitatin**G** the **A**uthorisation of **P**reparation **P**rocess for Blood, Tissues and Cells) of a 36months EU Joint Action started in May 2018. The main aim of **GAPP** is to facilitate the development of a common and optimal approach to assess and authorise preparation processes, in Blood and Tissues Establishments adapting requirements as prescribed by Article 29 of Directive 2002/98/EC and Article 28 of Directive 2004/23/EC. **GAPP** is giving particular attention to innovative processes under development and/or previously described in relevant EU Joint Actions.

GAPP is coordinated by the Italian National Institute of Health, National Transplant Centre, and National Blood Centre and includes 17 European countries and more than 40 organisations in the consortium.

GAPP INTERIM MEETING ROME

General

The interim meeting was attended by representatives of all the horizontal and technical work packages. It is important to note that the GAPP External Advisory Board (EAB) attended the interim meeting. The EAB is composed by one expert for Blood, Johanna Wiersum; one for Tissues, Johan Guns; one for HSC, Ineske Slaper Cortembach; one for Medical Assisted Reproduction (MAR), Kelly Tilleman; one expert in TTD testing, Ines Ushiro Lumb; one for clinical trials, Andrijana Tivadar; one microbiology expert, Veroniek Saegeman. Prior to the interim meeting, the EAB evaluated one technical deliverable - D8.1. Their expert assessment was shared with WP leaders present at the meeting, and is also available in the Intermediate evaluation report. Evolution of

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Brexit process is being monitored and solutions on the management of collaboration with UK partners in the future have been taken into account.

EU COMMISSION FOR GAPP

DG SANTE representative summarised the high-level findings discussed during the Evaluation process of EU directives and why GAPP JA is very important for the Commission. During the Evaluation report presentation, there was a broad consensus on the document (available at the link https://ec.europa.eu/health/sites/health/files/blood_tissues_organs/docs/swd_2019_376_en.pdf) summarising the evaluation of directives 2002/98 for Blood and 2004/23 for Tissues and Cells,

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performed with the main aim of assessing how the EU legislation improved the quality and safety issues, whether it achieved its original objectives and whether it is still fit for purpose.

The evaluation was expected to provide a sound evidence base which will be used to consider the need for any changes to the legislation.

INTERIM OVERVIEW ON TECHNICAL WP's

WP5 - Development of Overall Guidance on organisation of PPA system (Gerard Sheridan, HPRA, Ireland; Ruth Barrio, CatSalut, Spain)

Deliverable 5.1 aims at extending the Outputs of Previous Projects – VISTART, GTPII etc. to the Blood field. It will be finalised in April 2020 after the second multi-country workshop. So far WP5 have reviewed a Blood risk assessment and compared it with EUROGTP tool, the future discussion to be brought to the second workshop will be mostly focused on how to enlarge the EUROGTP II risk assessment tool also to the Blood group.

The preparation of D5.2 is ongoing and will describe the outcome of the Survey circulated in April 2019 (structured in three parts and issued to Competent Authorities in Blood, Tissues and Cells) and the desk-based review of PPA in other fields. The final Deliverable of WP5 will be the D5.3 Good Practice Guideline on the authorisation of preparation processes in Blood, Tissues and Cells (BTC) to be delivered at the end of the Action.

WP6 - Technical Annex 1 to overall guidance: authorisation of changes in donation, procurement and collection, processing, preservation, storage and distribution (Samuel Arrabal, Katia Bruneau, ABM, France)

The aim of WP6 is to prepare the first annex of the guideline, focusing on the authorisation of changes in donation, procurement and collection, processing, preservation, storage and distribution (including labeling and package inserts), taking into account that the quality of the preparation of BTC has an impact on patients and will condition the authorisation of preparation process steps. Within this WP there is the need to establish clear quality criteria for different blood components and tissues and cells.

The final deliverable should be ready by September 2020.

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The work of WP6 was divided in two parts:

Part 1 is focused on the Definition of the critical characteristics/properties for each category of blood components and tissues and cells. It will also highlight the criteria that need to be validated or verified through in vitro or by clinical studies (build on existing CoE EDQM, EU Blood legislation 2004/33/EC)

Part 2 is focused on defining Guidance on how to ensure these criteria are met through in vitro validation, in-process verification or clinical studies. (Assessment of new procedure's validations by using published studies and of laboratory studies conducted by the applying BE/TE as part of their validation, depth of validation for procedures already validated elsewhere, depth of validation for procedures not validated anywhere before).

The overall work in WP6 was divided in four subgroups: the one for BLOOD led by Imad Sandid (ANSM), MAR by Dominique Royere (ABM), TISSUES by Isabelle Martinache (ABM) and CELLS by Eoin McGrath (JACIE/EBMT).

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WP6 Expert Workshop Rome 30th October 2019

The WP6 technical meeting in Rome gave the opportunity for a collaborative work on a consensus for finalizing Part 1 of WP6 Technical Annex 1 to the Guideline.

WP7 - Technical Annex 2 to overall guidance: assessing the quality and safety of donor testing, pathogen reduction and sterilisation steps as part of PPA (Anu Puomila, FIMEA, Finland and Katia Bruneau, ABM, France)

WP7 has one single deliverable to be prepared by June 2020 and the focus of this annex is donor/donation testing, pathogen reduction and sterilisation of final products. Subgroup work was identified as follow: 1) Requirements for selection, validation and performance of donor/donation infectious marker testing kits and other methods; 2) Requirements and criteria for laboratories performing donation/donor infectious disease screening and microbiological testing of Blood components/Tissues/cell grafts; 3) Criteria for validation of pathogen reduction steps ; 4) Criteria for validation of sterilisation processes; 5) Requirements and criteria for microbiological quality of the final product.

Milestone 27 (Detailed plan of the Deliverable Technical annex on assessing the quality and safety of donor/donation testing, pathogen reduction and sterilization steps as part of PPA agreed on) was achieved in April 2019 and according to this plan the subgroups are working and will meet face-to-face for a technical workshop in Langen, Germany on 27th February.

The drafting of the deliverable is ongoing and the final draft will be shared for comment in April 2020 and finalized in June 2020.

WP8 - Technical Annex 3 to overall guidance: assessing clinical data as part of PPA authorisation (Anu Puomila, FIMEA, Finland; Jaime Tabera, BST, Spain)

WP8 is focused on the assessing of clinical data as part of the PPA authorisation.

The first **Deliverable 8.1** associated to this WP (Defining the current state of the art of existing collected clinical data) was submitted to EC evaluation on the participant portal. To prepare this deliverable the main European Tissue & Cell registries were interviewed namely: i) EBMT (Hematopoietic Stem Cell Transplants & Cell Therapies), ii) ECCTR (Cornea & Cell Transplantation), iii) ESHRE (MAR). Two national registries the National registry on fertility treatments (THL,

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Finland) and the Registo Português de Transplantação on Donation, Procurement, Transplant and Follow up of Organs, Tissues and Cells (RPT, Portugal). According to the evaluation conducted it was concluded that (i) registries collect the most relevant clinical indicators defined by clinicians and experts of the sector in question; (ii) at present time only EBMT and ECCTR hold individual patients' data, whereas other SoHO sectors do not have such detailed data; (iii) objectives of the registries are to promote scientific knowledge and to assess the efficacy of the different therapies amongst the stakeholders; (iv) there are some limitations for regulatory purposes (e.g. level of detail and completeness of data for different BTCs); (v) Tissue & Cell registries are considered valuable and useful for the future development of the work foreseen by the WP8.

Deliverable 8.2. Defining a risk-based set of criteria to assess whether the current clinical data fit to a new processing or testing protocols for Blood, Tissues and Cells therapeutics is undergoing the evaluation of the EAB and after collecting their feedback will be submitted on the participant portal for EC evaluation.

Deliverable D8.3 (Methodological framework to evaluate quality and safety of human Blood, Cell, and Tissue therapeutics based on clinical outcome data requested for authorisation processes upon introduction of innovation to the current processing and testing protocols) will be prepared and the work divided in Expert groups. Next WP8 meeting was scheduled for Feb 5th 2020 in Brussels.

Deliverable D8.4 (Data model of information on clinical outcome of application of human Blood, Cell, and Tissue therapeutics) will be prepared mostly in co-operation with WP9 leader Paul-Ehrlich-Institut, DE but also with WP5, 6 and 7.

WP 9 - Knowledge sharing on PPA between EU CAs (Winfried Kammer, PEI, Germany)

WP9 will develop the knowledge sharing database. The tool will support the complete assessment of PPA by providing/sharing information and increasing efficiency.

The main expectation of the tool will be: 1) Supporting assessors; 2) Harmonization and Standardization (by use); 3) Balancing benefit and risk; 4) Supporting assessment of novel PPA; 5) Direct assessment information; 6) Assessment algorithms and matrices (to be discussed); 7) Data from application; 8) Clinical outcome data (prepared); 9) Kind of a tool box?; 10) Data sharing platform; 11) Knowledge sharing platform; 12) User-friendliness; 13) Ergonomic software; 14) Flexibility / Adaptability.

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INTERIM DISSEMINATION KPI's

WP2 (Dissemination & Communication) aims at maximizing the impact of GAPP and its visibility also after the end of the action. WP2 leaders encouraged all the partners to be responsible for the dissemination of GAPP in their countries. The dissemination strategy was submitted at the very beginning and recently revised according to some KPIs.

Apart from the project's website with it public and private area an application for mobile phone has recently been developed with a direct access of social media account of the project. The layman brochure and newsletters have been prepared and disseminated to all identified relevant stakeholders and a big effort has been done to have the presence of GAPP in scientific association webpages, one example is EHA.

According to analytics data, the GAPP website registered 1612 unique users, for a total number of 2839 visits, 9182 Pageviews and 4:04 Avg Duration of each session.

Future plans of WP2 are: i) to Reach the widest possible audience by applying all available dissemination tools; ii) Close monitoring of dissemination KPIs and update strategy accordingly; iii) Active involvement of all JA participants in the dissemination activities; iv) Focus on the publication strategy.

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Where can you meet GAPP in the forthcoming international congress?

EBMT – conference booth: Madrid, March 22nd -25th https://www.ebmt.org/annual-meeting

ESHRE congress – European Commission conference booth: Copenhagen, July 5th – 8th https://www.eshre.eu/eshre2020

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