Research Networking Programmes

Science Meeting – Scientific Report

Scientific report (one single document in WORD or PDF file) should be submitted online within two months of the event. It should not exceed seven A4 pages.

***Proposal Title**: European Interdisciplinary Summit on Cell-Based ATMPs*

Application Reference N°: 4831

1) Summary (up to one page)

The **European Interdisciplinary Summit on Cell-Based ATMPs** was organized by the Chair, the External Coordination, and a Professional Congress Organizing Bureau, and took place on May 02-03 at the Billroth-Haus in Vienna. The intention behind this 2-day meeting was to provide a novel forum for the in-depth assessment and discussion of the challenges involved in the development, application and marketing of cell-based ATMPs in this dynamic and fast moving field, with special emphasis on regulatory issues and safety models. The meeting was attended by 75 persons, 60 participants from academia, 16 from industry, and 9 from regulatory authorities. We were able to put together a very good programme, covering the main issues identified at the kick-off meeting in Frankfurt. The latter kick-off meeting was organized to discuss the format, ideas, structure and topics of the final REMEDIC conference. Overall, the Summit was a full success, and we received many enthusiastic comments about this meeting format. Bringing together academia, industry, and regulators in this rapidly evolving field is an obvious need in Europe. The Summit established strong ties between European industry and REMEDIC, and led to tangible results. First, we were able to put together a White paper summarizing the meeting outcome. We are indeed very proud of this exceptional document jointly authored by academic researchers, company representatives, and regulators. Secondly, although the final decision is still pending, it was considered to organize similar follow-up summits involving academia, industry, and regulators back-to-back with the yearly meeting of the Alliance for Advanced Therapies.

2) Description of the scientific content of and discussions at the event (up to four pages)

The purpose of organizing the final REMEDIC conference on regulations, rules and standards in regenerative medicine entitled "*European Interdisciplinary Summit on Cell-Based ATMPs*" on May 02 – 03 in Vienna was to bring together leading academic experts in the field of cell-based Advanced Medicinal Therapy Products, European and national regulators, company representatives, representatives of tissue banks, but also young scientists. The intention behind this 2-day meeting was to provide a novel forum for the in-depth assessment and discussion of the challenges involved in the development, application and marketing of cell-based ATMPs in this dynamic and fast moving field, with special emphasis on regulatory issues and safety models.

To discuss the format, ideas, structure and topics of this final REMEDIC conference we had organized a **kick-off meeting** at the Frankfurt Airport Centre on June 04, 2012. Representative from ESF (Kirsten Steinhausen), REMEDIC (Reinhold Erben, Frank Luyten, Gustav Steinhoff, Gudrun Tiedemann, Enrique Gomez-Barrena, Eva Sykova), the Alliance for Advanced Therapies (AAT, Alexander Vos), and CAT (Martina Schüssler-Lenz) took part in this meeting. It was generally felt that a European conference on regulatory issues in the ATMP field was very timely, and would fill an important gap in Europe. During the kick-off meeting, the following important needs were identified which were later addressed in the final REMEDIC conference.

- More interaction between disciplines
- Reimbursement & treatment algorithms
- Development of non-clinical efficacy and safety models
- Improved safety models, especially for tumorigenesis (long-term safety)
- Increase treatment success by removing boundaries between academia and industry
- Models for regulation of IPR issues between academia and industry
- Manufacturing challenges
- Roadmap for ATMPs
- More focus on personalized medicine
- Quality assurance in multi-centre settings
- Better educational models
- European post-marketing registry
- Better strategies to deal with ethical issues

The **European Interdisciplinary Summit on Cell-Based ATMPs** was organized by the Chair, the External Coordination, and a Professional Congress Organizing Bureau, and took place on May 02-03 at the Billroth-Haus in Vienna. The Billroth-Haus, named after the famous surgeon Theodor Billroth, was picked as a meeting place because this historic building was one of the centres of medical progress in the 19th and early 20th century. The meeting was attended by 75 persons, 60 participants from academia, 16 from industry, and 9 from regulatory authorities. We were able to put together a very good programme, covering the main issues identified at the kick-off meeting in Frankfurt.

The following topics were addressed during the 2-day meeting:

EU Research Agenda, Interdisciplinarity & Education

Treatment Algorithms and Reimbursement

Manufacturing Challenges

Non-Clinical Models. Strengths and Limitations

Safety models/Tumorigenesis (long-term safety)

Ethics and Economy

Clinical studies and Quality Assurance

Roadmap 2020 for ATMPs. This last session war organized as a panel discussion with participants from academia, companies, and regulatory authorities. Panel members were Frank Luyten, Leuven (B), Gustav Steinhoff, Rostock (GER), Katarina LeBlanc, Stockholm (S), Wilfried Dalemans, Leuven (B), Michaela Kneissel, Basel (CH), Alexander Vos, Maastricht (NL), Martina Schüssler-Lenz, Langen (GER), Beatriz Silva Lima, Lisbon (P)

In the session on EU Research Agenda, Interdisciplinarity & Education, we were honoured by the presence of Arnd Hoeveler, Head of Unit at the European Commission, who gave a lecture about "*The EU research agenda in cell therapy: opportunities and challenges*".

Overall, the Summit was a full success, and I received many enthusiastic comments about this meeting format. Bringing together academia, industry, and regulators in this rapidly evolving field is an obvious need in Europe.

A very important outcome of the Summit was a **White paper on how to advance cell-based Advanced Therapies in Europe** covering the following topics:

- National hurdles, harmonization
- Hospital exemption, national and subnational differences
- Reimbursement
- Knowledge on the mode of action
- Predictive preclinical efficacy and safety testing
- Need for innovative systems for preclinical testing
- Product characterization and product potency
- Manufacturing with cost of goods in mind
- Clinical trials: design and outcomes

The White paper is a summary of the discussions and exchange of experiences during the first European Interdisciplinary Summit on Cell-Based ATMPs, and is jointly authored by

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Another positive aspect of the Summit underscoring its visibility and importance is that the MRC, although not part of the national funding bodies supporting REMEDIC, decided in March 2013 to additionally support our final REMEDIC conference with 10,000 £.

3) Assessment of the results and impact of the event on the future directions of the field (up to two pages)

The Summit established strong ties between European industry and REMEDIC, and led to tangible results. First, we were able to put together a White paper summarizing the meeting outcome. We are indeed very proud of this exceptional document jointly authored by academic researchers, company representatives, and regulators. The White paper may have a large impact on the further development of the ATMP field in Europe. Secondly, although the final decision is still pending, it was considered to organize similar follow-up summits involving academia, industry, and regulators back-to-back with the yearly meeting of the AAT. This decision will be made in the next weeks during a conference call between the management of AAT and the Chair of REMEDIC. Therefore, the spirit of REMEDIC may live on.

4) Annexes 4a) and 4b): Programme of the meeting and full list of speakers and participants

Annex 4a: Programme of the meeting

Program_ESF2013_0429.pdf

Annex 4b: Full list of speakers and participants

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