Gene and Cell Therapy Funding Opportunities in Horizon 2020: An Overview for 2014–2015

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SINCE 1984, THE EUROPEAN COMMISSION HAS undertaken seven framework programs (FPs), dedicated to research and innovation, that ran for 5–7-year periods. During the fifth, sixth, and seventh FPs, more than €351 million has been invested in 110 projects in the gene transfer and gene therapy field (Table 1). While in FP5 and FP6, support was allocated mostly to small projects addressing specific problems such as refining targeted gene delivery or transgene expression in specific cells, the large-scale collaborative projects of FP6 and FP7 brought this research field from bench to bedside by gathering the critical mass of scientific expertise.

Presently, 15 FP7 gene therapy projects include clinical trials mostly for patients suffering from a rare disease or cancer, but also from more frequent disorders such as severe fetal growth restriction or age-related macular degeneration. Some of those projects (as well as their participants) derive directly from research and technologies developed in previous FPs. For example, the projects Cell-PID, performing clinical trials for primary immunodeficiencies (PIDs), and SUPERSIST, developing scale-up production of targeted genetic correction of hematopoietic stem cells and T cells for PIDs and leukemia, both derive from former FP7 PER-SIST (refinement of gene therapy vectors for persisting transgenesis) and FP6 CONCERT (safety and efficiency evaluation of retroviral transgenesis) collaborative projects (ec.europa.eu/research/health/biotechnology/new-therapies/projects-fp7-gt_en.html). The specificity of these collaborative grants lies in gathering international expertise in various research disciplines, from scale-up and good manufacturing practice, development of new preclinical models, and toxicology and monitoring tests to regulatory and ethical issues of clinical trials.

The eighth research and innovation program, Horizon 2020 (2014–2020), is conceived as one of the central means to drive economic growth and job creation by addressing the entire innovation cycle, from basic research to implementa-

tion. In structure, Horizon 2020 has three main pillars: excellence in science, industrial leadership, and societal challenges. The challenge addressing primarily health research under the new program will have a slightly larger budget, €7.4 bn, compared with previous programs (€2.65 bn in FP6 and €6.2 bn in FP7). The first calls for societal challenges of “health, demographic change, and wellbeing” are published. Applicants are expected to focus on problem solving, and therefore gene and cell therapy projects could find funding opportunities in topics advertising chronic or rare disease research as well as regenerative medicine or advanced therapies (Table 2).

The topic “clinical research on regenerative medicine” will be open for 2 years, providing sequential or iterative opportunities to proposals ready to test in the clinic new regenerative applications. The field has considerably developed during recent years and is now ripe for clinical challenges. The topics on rare diseases (one in 2014, two in 2015) will support the general objectives of the International Rare Diseases Research Consortium (IRDiRC), initially

TABLE 1. EUROPEAN UNION SUPPORT TO GENE TRANSFER AND GENE THERAPY PROJECTS OVER THE YEARS^a

<i>Program (period covered)</i>	<i>Number of projects</i>	<i>EU contribution (million EUR)</i>
FP5 (1998–2002)	29	48.9
FP6 (2002–2007)	27	98.8
FP7 (2007–2013)	28 + 26 (ERC)	154.2 + 49.2 (ERC)
Total	110	351.1

ERC, European Research Council; FP, framework program.

^aThese numbers do not take into account gene transfer projects for vaccination purposes, the European training grants, or the projects in which gene transfer represent a minor part of the work, such as generation of induced pluripotent stem (iPS) cells.

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TABLE 2. SELECTION OF HORIZON 2020 FUNDING OPPORTUNITIES FOR GENE AND CELL THERAPY PROJECTS

<i>Topics from societal challenges</i>	<i>Specific challenge</i>	<i>Scope summary</i>
2014/2015—Clinical research on regenerative medicine	Specific research is needed for proving safety, efficacy, and repeatability of new treatments in regenerative medicine clinical trials.	Proposals should focus on regenerative medicine therapies ready for clinical (in-patient) research and may address any disease or condition. Proposers should have or be close to have the necessary ethical and regulatory authorizations to carry out the work.
2014—New therapies for chronic noncommunicable diseases	Development of new therapies is stagnating because of lack of clinical validation.	Clinical trial(s) supporting proof of concept of novel therapeutic concept(s) and/or optimization of available therapies. Application may build on preexisting preclinical research and results from databases.
2014—ERA NET: Rare disease research implementing IRDiRC objectives	In the area of rare diseases, promote transnational cooperation and pooling of resources while developing common standards and research priorities.	Feasibility assessment and effectiveness should be provided if relevant. Proposals should coordinate national and regional programs for research on rare diseases to implement IRDiRC objectives. Further the understanding of disease mechanisms and natural history of rare diseases with objective to develop new diagnostic tools and treatments. Proposals should be complementary with other funding programs and activities on European and international level.
2015—New therapies for rare diseases	Specific problems posed in rare disease therapy development include the following: small and dispersed patient populations nature of the therapies proposed regulatory hurdles limited market providing low commercial return	Proposals may address one or more of the following: development of new or improved therapeutic approaches, from small molecule to gene or cell therapy repurposing of existing therapies preclinical research, animal model development, and GMP production Clinical trials only supported in cases where “orphan designation” has been given and where its design takes into account recommendations given by the European Medicines Agency, including a clear patient recruitment strategy. Considerations of effectiveness/potential clinical benefit should be integrated in the application if relevant. Selected proposals should contribute to the objectives of and follow the guidelines and policies of IRDiRC.
2015—Tools and technologies for advanced therapies	New therapies, such as gene or cell therapies, tissue engineering, or regenerative medicine often require technological innovation to bring these innovative treatments to the patient and achieving regulatory compliance.	Proposals should focus on refining a particular technological step or component needed by the therapeutic approach. Establishing proof of concept for the new technology and carrying out preclinical research may be included if needed. Regulatory aspects of the new technology should be addressed as appropriate.
2015—Support for the European Reference Networks: Efficient network modeling and validation	Centres of Expertise will be able to collaborate, coordinate, and share their knowledge across borders. The demonstration and validation project of ERNs as an innovative model of cooperation and work between highly specialized health-care providers is required.	Proposals should provide coordinated support to the activities of the ERNs under the framework of a directive concerning validation of a model for the optimal organization, governance, maintenance, and continuous monitoring and evaluation of the ERNs and their centers.

ERNs, European reference networks; IRDiRC, International Rare Diseases Research Consortium.

established by the European Commission and the U.S. National Institutes for Health Research in 2011. The consortium aims at developing 200 new therapies and the means to diagnose most rare diseases by 2020. The challenges at stake are too ambitious for any country to master alone; therefore, IRDiRC now counts more than 35 funding members from four continents (www.irdirc.org/).

Included in the same call, a support action for European reference networks promotes cross-border cooperation among member states. These networks are meant to improve access to and provision of high-quality specialized health care to patients and to act as focal points for medical training and research and information dissemination and evaluation, especially for rare diseases. The topics “tools and technologies for advanced therapies” and “therapies for chronic noncommunicable diseases” offer as well interesting opportunities for innovation in the gene and cell therapy field. Moreover, the first pillar of Horizon 2020 offers opportunities for frontier research science via its European Research Council grants (erc.europa.eu/funding-and-grants), for international training via its Marie Skłodowska-Curie actions (ec.europa.eu/programmes/horizon2020/en/h2020-section/marie-sk%C5%82odowska-curie-actions) and for development of international infrastructure platforms (which explicitly mention the rare disease research) and key enabling technologies.

The second pillar will ensure support to research and innovation performers, including significant, tailored support to small and medium-sized enterprises for which a dedicated instrument has been developed (ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020/index.html). This is complementary to the Innovative Medicines Initiative (IMI; www.imi.europa.eu), Europe’s largest public–private partnership between the Commission and the European Federation of Pharmaceutical Industries and Associations aiming at promoting the development of new, improved diagnostics and therapies for patients. A renewed IMI with a budget of €3.5 bn is forecasted for the period 2014–2020.

More than ever, the European Union offers to the gene and cell therapy sector possibilities of financial support to bright and innovative consortia ready to develop, possibly in collaboration with the industry, new therapeutic applications to be tested in clinical trials or novel products for the market, and build sustainable networks of expertise in the field.

Recently, the first human gene therapy product in Europe, Glybera, entered the market (www.uniqure.com/products/glybera). It constitutes one of the four advanced therapy medicinal products approved by the European Medicine Agency since 2009 (www.uniqure.com/news/167/182/uniQure-s-Glybera-First-Gene-Therapy-Approved-by-European-Commission.html). Clearly, the field benefits, after a long period of stagnation, from renewed interest from academic and industrial stakeholders, including big pharma, and will witness exciting developments in the near future.

Author Disclosure Statement

The authors declare that no conflicts of interest exist.

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