Personalising health and care

H2020-PHC-2015-two-stageSub call of: H2020-PHC-2014-2015

Publication date	2013-12-11	**Deadline Date**	2014-10-14 17:00:00 (Brussels local time)
		Stage 2	2015-02-24 +17:00:00 (Brussels local time)
Budget	€330,000,000	**Main Pillar**	Societal Challenges
Status		**OJ reference**	OJ C 361 of 11 December 2013

Topic: New therapies for rare diseases PHC-14-2015

- Topic Description
- Topic Conditions & Documents
- Submission Service

Specific challenge: A considerable amount of knowledge has been generated by biomedical research in recent years, yet most of the 6000-8000 rare diseases are lacking therapies despite many diseases being life-threatening or chronically debilitating.

Specific problems posed in therapy development for rare diseases include the small and dispersed patient populations and the nature of the therapies proposed which are often highly specialised and novel requiring the engagement of regulatory authorities during development. In addition the limited market for such therapies provides a low commercial return.

Scope: Proposals may address one or more of the following: development of new or improved therapeutic approaches, for repurposing of existing therapies, as well as for preclinical research, animal model development and good manufacturing practice (GMP) production.

Proposed treatments to be developed may range from small molecule to gene or cell therapy.

Clinical trials shall only be supported in cases where "orphan designation" has been given and where the proposed clinical trial design takes into account recommendations from protocol assistance given by the European Medicines Agency and where a clear patient recruitment strategy is presented. The orphan medicinal product must have been granted the EU orphan designation[1] at the latest on the date of the call closure. A concise feasibility assessment justified by available published and preliminary results and supporting data shall also to be provided. 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 the International Rare Diseases Research Consortium, IRDiRC.

The Commission considers that proposals requesting a contribution from the EU of between EUR 4 and 6 million would allow this specific challenge to be addressed appropriately. Nonetheless, this does not preclude submission and selection of proposals requesting other amounts.

Expected impact: This should provide:

- Advancing the development of new therapeutic options for patients living with rare diseases.
- In line with the Union's strategy for international cooperation in research and innovation[2], proposals should contribute to reaching the IRDiRC objective to deliver 200 new therapies for rare diseases by 2020.

Type of action: Research and innovation actions

[1] The European register of designated Orphan Medicinal Products is available fromhttp://ec.europa.eu/health/documents/community-register/html/alforphreg.htm

[2] COM(2012)497