



UPGRADE Project

Unlocking Precision Gene Therapy

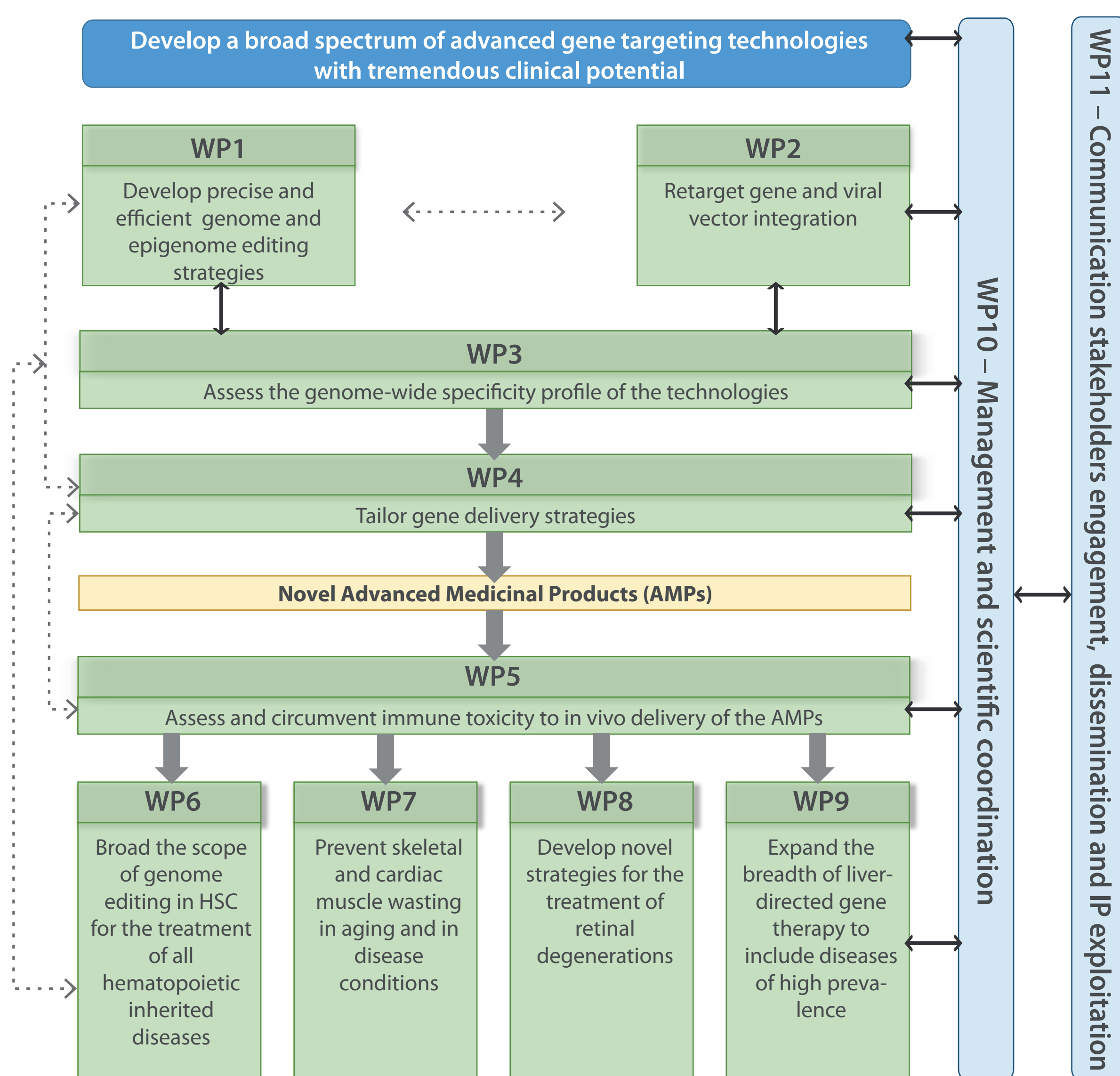


The overall goal of UPGRADE is to overcome current limitations that are hindering broad application of gene therapy, improving the safety, efficiency and in vivo delivery of advanced medicinal products (AMPs) based on gene and epigenome editing.

UPGRADE offers radical new solutions to the hurdles that currently limit efficacy and safety of conventional and emerging gene therapy approaches.

UPGRADE objectives will be achieved through the activities carried out within ten operational workpackages, besides the management and ethics ones:

Novel strategies to increase precision and efficacy of gene and epigenetic editing (**WP1**), Retargeting gene/vector integration (**WP2**), Genome-wide off-target profiling of gene-editing reagents (**WP3**), Viral and non-viral mediated delivery of genes and genome engineering components (**WP4**), Increase immune evasion of genome engineering components (**WP5**), Advanced genetic engineering of hematopoiesis (**WP6**), Novel strategies to target skeletal and cardiac muscle wasting in aging and disease conditions (**WP7**), Retinal degeneration (**WP8**), Expanding the breadth of liver directed gene therapy to include larger patient populations (**WP9**), Communication, stakeholders engagement, dissemination and exploitation (**WP11**).



UPGRADE Publications

In the first year, UPGRADE Consortium published 4 papers in high-ranked journals. All publications are available at <http://www.upgrade-h2020.eu/publications/>

UPGRADE Consortium

- Fondazione Telethon, Italy
- Ludwig-Maximiliansuniversitaet Muenchen, Germany
- Massachusetts General Hospital, United States
- Pompeu Fabra University, Spain

- Technische Universitaet Dresden, Germany
- University of Nantes, France
- University of Trento, Italy
- Vrije Universiteit Brussel, Belgium

UPGRADE multidisciplinary consortium includes experts in precision gene editing, gene and cell therapy and biotechnology

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