SCIDNET website:
www.scidnet.eu

Project Coordinator
Prof Bobby Gaspar
h.gaspar@ucl.ac.uk

FOR IMMEDIATE RELEASE

The SCIDNET European research project will develop genetic medicines for severe combined Immunodeficiency

London, 11 May, 2016 – A European Commission-funded project is looking to offer gene therapy as a curative option for over 80% of all forms of SCID in Europe allowing the affected children to live normal functional lives.

Severe combined immunodeficiency (SCID) is a devastating rare disorder of immune system development. Affected infants are born without functional immune systems and die within the first year of life unless effective treatment is given. Treatment options are limited to allogeneic haematopoietic stem cell transplantation and autologous stem cell gene therapy. Over the last 15 years, gene therapy for two forms of SCID (SCID-X1 and ADA SCID) has shown significant safety and efficacy in correcting the immunodeficiency and allowing children to live normal lives. Proof of concept of gene therapy for 3 other SCID forms has also been shown by members of the proposed SCIDNET consortium and is ready for translation into clinical trials. We are therefore in a position whereby, over the next 4 years, we can offer gene therapy as a curative option for over 80% of all forms of SCID in Europe. In addition, we will investigate the future technologies that will improve the safety and efficacy of gene therapy for SCID. Our proposal addresses an unmet clinical need in SCID, which is classified as a rare disease according to EU criteria (EC regulation No. 141/2000). The proposal also addresses the need to develop an innovative treatment such as gene therapy from early clinical trials though to a licensed medicinal product through involvement with industry, regulatory agencies and is in keeping with the ambitions of the IRDiRC. The lead ADA SCID programme has Orphan Drug Designation and clinical trial design is assisted by engagement with the European medicines Agency. The ADA SCID trial will act as a paradigm for the development of the technologies and processes that will allow gene therapy for not only SCID, but also other bone marrow disorders, to become authorised genetic medicines in the future

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement SCIDNET (No 666908)

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About the Project Partners

UNIVERSITY COLLEGE LONDON (United Kingdom) – www.ucl.ac.uk

The UCL Institute of Child Health (www.ucl.ac.uk/ich) with its clinical partner Great Ormond Street Hospital for Children (GOSH), forms the largest concentration of children's health research in Europe. UCL ICH staff includes 52 full professors (35 clinical and 17 non clinical); 13 readers; 5 clinical senior lecturers; 12 non clinical senior lecturers and 7 lecturers. Currently there are 175 PhD and masters students in training. There are 5 academic research programmes and the UCL ICH also organises a number of Masters Programme including a specific Masters programme in gene and Cell therapy. Research grants currently total in excess of £30 million per year providing support for Great Ormond Street Hospital / Institute of Child Health's research portfolio of approx. 800 ongoing research Projects of which approx. 600 are classified as clinical or translational research.

Leiden University Medical Center (The Netherlands) - www.lumc.nl

Internationally renowned biomedical research institute incorporating all activities of the Medical Faculty of Leiden University as well as its University Hospital. The LUMC, which employs nearly 7000 people, is one the few academic medical centres in the Netherlands where the core activities include fundamental research. The interaction between fundamental research and patient care forms the basis of its success. LUMC has a major scientific role as a tester of new and existing medical technologies. The LUMC is one of the leading centres in the Netherlands with respect to stem cell transplantation and solid organ transplantation. The LUMC produces over 100 PhD thesis and 1800 publication annually. Each year the LUMC welcomes many visiting scientists and foreign PhD students, offering a top notch and inspirational environment for scientists and clinicians alike.

French National Institute of Health and Medical Research (France) - www.inserm.fr

Founded in 1964, the French National Institute of Health and Medical Research (INSERM) is a public scientific and technological public research institute that operates under the joint authority of the French Ministry of Health and French Ministry of Research. One of INSERM mission is to focus on human health and as a consequence, in 2008 INSERM took on the responsibility for the strategic, scientific and operational coordination of biomedical research with the establishment of Clinical Research Centers (CIC). INSERM, which is a partner for SCIDNET, is composed of two entities: the Human Lympho-Hematopoeisis Laboratory in Imagine Institute and the Clinical Investigation Research Center of Biotherapy, both localized on Necker-Sick Children Hospital Campus (Paris, France). Imagine Institute and Necker Hospital are both research and innovative healthcare institutes dedicated to a better understanding of genetic diseases for pediatric patients. More than 800 scientists, physicians, engineers, technicians and healthcare professionals work together to bring faster to patients and their families the diagnostic and therapeutic solutions for an innovative and specialized healthcare.

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GeneWerk GmbH (Germany) - www.genewerk.de

GENEWERK is a SME and was founded as a spin-off of the German Cancer Research Center (DKFZ), Heidelberg, Germany in 2014 by C. von Kalle, M. Schmidt, A. Deichmann and DKFZ. The team has 20 years of experience in the area of hematology, oncology, virology and vectorology with focus on integration site analysis, high throughput sequencing, whole genome sequencing and bioinformatics. The invention and further development of Linear Amplification-Mediated PCR (LAM-PCR) by Manfred Schmidt and Christof von Kalle allows the highly sensitive identification and sequencing of unknown, flanking DNA or RNA sequences, which became the gold standard in gene therapy vector safety studies. GeneWerk GmbH provides custom-tailored services for the assessment of the safety of viral vectors in the context of gene therapy trials, for determination of the immune cell receptor repertoire and for the development of bioinformatic tools for NGS sequence analyses.

Ospedale San Raffaele (Italy) - www.hsr.it

OSR is one of the leading private scientific research institutes in Italy, recognized by the Italian Ministry of Health as a Research Hospital. It comprises both clinical and research activities, conducted by a highly specialized and qualified hospital and a research institute with clinical and translational scientists. The premises also host the Vita-Salute San Raffaele University, which comprises the faculties of Medicine, Psychology and Philosophy and provides specialized post-graduate courses, residency programs and international PhD programs. OSR performs cutting-edge science and aims at advancing the knowledge about human diseases and novel therapies through translational research. In particular OSR expertise-excellence areas are: Oncology; Neuroscience; Metabolic and Cardiovascular Sciences; Regenerative Medicine, stem cells and gene therapy; Immunology, Transplantation and Infectious Diseases; Genetics and Cell Biology; Genomics, Bioinformatics and Imaging technologies.

Medical Center – University of Freiburg (Germany) www.uniklinik-freiburg.de

UKLFR is one of the largest medical centers in Europe, with representations of all medical specialties. The medical faculty is part of the Albert-Ludwigs-University of Freiburg, which has put forward Immunology and Infectious Diseases as one of the five strategically supported research areas of the Medical Center. In order to support Freiburg's leading role in immunology, the Federal Ministry of Education and Research has funded the setup of the Center for Chronic Immunodeficiencies (CCI) in 2008 and the Institute for Cell and Gene Therapy (IZG) in 2013. The IZG combines an established facility for diagnostic services with specialized personnel for GMP-compliant manufacturing processes, and research groups focusing on (i) the improvement of safe genome editing tools (incl. TALENs, CRISPR-Cas) for therapeutic applications in human stem cells, (ii) the development of disease models and cell therapies based on induced pluripotent stem cells, and (iii) the translation of genome editing for rare immunodeficiencies, HIV infection, and leukemia.

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Association Genethon (France) - www.genethon.fr

Genethon is a non-profit, translational R&D organization created in 1990 and funded by the Association Française contre les Myopathies.The mission of GNT is to develop gene therapies for genetic diseases. With over 230 scientists, physicians, engineers and regulatory affairs specialists, GNT develops gene therapy products based on lentiviral vectors (LV), adeno-associated (AAV) vectors and genetically modified hematopoietic stem cells from vector design to pre-clinical proof of concept, efficacy and safety, including process and analytical development. GNT is developing a pipeline of gene therapies at both preclinical and clinical level in the areas of hematopoietic, neuromuscular, eye and liver diseases. GNT currently hosts 12 R&D units, 4 technology platforms/services, a department of medical/regulatory affairs, and a business/intellectual property unit. GNT hosts a unit of the INSERM, the French National Research Institute, which provides academic affiliation to scientists and access to training, technological platforms, and resources.

Hannover Medical School (Germany) - www.mh-hannover.de

Hannover Medical School (MHH) is one of the biggest centres for transplantation medicine in Germany and among the 5 top university hospitals in Germany. MHH's top priorities are transplantation, immunity & infection, regeneration, biomedicine technologies and bioimplants. MHH has an excellent campus combining state of the art hospital, teaching and research facilities. Several core units, including next generation sequencing, transcriptomics, confocal microscopy, proteomics, FACS sorting and imaging support the research activities. The focus on transplantation, immunity and regeneration is further underlined by the fact that MHH hosts big network clusters of excellence, such as the BMBF-funded integrated research and treatment centre transplantation (IFB-Tx) and the DFG-funded cluster of excellence REBIRTH (from regenerative biology to reconstructive therapy).

Great Ormond Street Hospital (United Kingdom) - www.gosh.nhs.uk

GOSH's mission is to provide world-class clinical care and training, pioneering new research and treatments, in partnership with others, for the benefit of children in the UK and worldwide. Across our departments and divisions we have a shared vision to produce world leading translational and patient-orientated research. We are always aspiring to develop new research and treatments across the breadth of paediatric specialities. Great Ormond Street Hospital for Children is fully committed to conducting high quality research in collaboration with our various academic and industry partners. GOSH is one of two centres in the UK designated to look after patients with SCID. As the larger of the two centres, GOSH sees more children with SCID than any other hospital in the UK. GOSH is also a major international referral centre for primary immunodeficiency and has a very strong bone marrow transplant programme for SCID. Through its collaboration with UCL GOSH has treated more children by gene therapy for primary immunodeficiency than any other centre worldwide.

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Miltenyi Biotec (Germany) - www.miltenyibiotec.com

Since its foundation in 1989 Miltenyi Biotec has become one of Germany's most successful biotechnology companies with currently more than 1.500 employees worldwide. Miltenyi's multidisciplinary R&D department, consisting of 350 scientists, technicians and engineers, is constantly developing novel reagents and instruments for use in immunology, cell biology and molecular biology. Magnetic Cell Sorting Technology (MACS®) from Miltenyi has become an established standard method in biomedical research areas like cancer research, hematology, stem cell biology or neurosciences, and furthermore in novel clinical approaches such as cellular immunotherapy, transplantation or regenerative medicine. With MACS Technology, virtually any cell type can be isolated from human and animal cells up to plant cells and bacteria. Miltenyi Biotec develops, produces and markets more than 15.000 state-of-the-art products and services.

Centre Hospitalier Universitaire Vaudois (Switzerland) - www.chuv.ch

For the past 16 years, Dr. Candotti has led a research group in the field of gene therapy that has performed preclinical development of a series of viral vectors for the correction of several primary immunodeficiencies (PIDs) including SCIDX-1, JAK3-SCID, ADA-SCID, IL12Rb1-deficiency, and the Wiskott-Aldrich syndrome (WAS). In addition, Dr. Candotti has co-developed a series of 3 gene therapy trials for ADA-SCID using retroviral vectors and contributed to the development of an ongoing trial of lentiviral gene therapy for the same disease. He has published ~30 articles in the area of gene therapy for PIDs. Dr. Candotti's team research team has recently applied zinc finger nuclease and CRISPR/Cas-9 technologies to target the WAS locus in mice (Mol Ther 2014 22, S216-S217) using approaches similar to the ones proposed in the grant application. Technical/scientific added value that the CHUV will bring to the proposed project as a partner. In addition to the experience in gene transfer, Dr. Candotti's group has extensive expertise in the cellular and biochemical aspects of ADA deficiency that will be required for the execution of the proposed tasks.

The International Patient Organisation for Primary Immunodeficiencies (United Kingdom) - www.ipopi.org

IPOPI, the International Patient Organisation for Primary Immunodeficiencies, is the Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency (PID) patients worldwide Established in 1992, IPOPI works as the global advocate for the PID patient community in cooperation with its National Member Organisations (NMOs) and key PID stakeholders.

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