





CeMM and Haplogen make available the world's largest collection of engineered human cell lines for biomedical discovery

Vienna, Europe, August 26, 2013 - Haplogen, a biotechnology company developing antiviral therapies, and CeMM, the Center for Molecular Medicine of the Austrian Academy of Sciences, today announced that they are making available their large collection of human cell lines that are deficient for single genes, which they have been building over the past three years as part of a public-private partnership. The partnership, through Haplogen, will distribute requested cell lines to the research community. The collection and the technological advances that enabled its development were published in *Nature Methods** on August 25. It currently includes cell line clones covering 3,000 different human genes, which represents about one third of all the genes that are active in these cells. The collection will continue to expand until all the genes have been targeted.

Although cell lines of human origin have been around for many years, they are all vastly different from each other, making them very difficult to control when performing genetic experiments, thereby limiting their use particularly for drug discovery efforts and discovering the function of genes. This new collection circumvents this problem by providing individual gene mutations in an otherwise identical genetic background.

Prof. Giulio Superti-Furga, Director of the CeMM who initiated this project commented: "This collection will fuel research in molecular medicine where the vast majority of human genes remain poorly understood and await functional characterization. Obtaining human cells where an individual gene is inactivated has so far been difficult and very tedious. With this largest human cell line collection available to date we expect to drive countless scientific discoveries in the research community."

"The creation of those precise mutants has become possible by the use of a haploid cell line. Destroying a single gene in haploid cells will immediately cause a detectable change – in contrast to our natural cells that always bear two copies of each gene" explains Thijn Brummelkamp, the inventor of the haploid genetics technology in human cells that was used to create this collection, and founder of Haplogen.

Georg Casari, CEO of Haplogen: "In this publication we show that these cell lines really behave as if only the gene of choice is no longer present. We have taken great care to document that the gene products are gone and that those clones have new properties as compared to the parental, unmodified cell line. We are excited to provide and distribute this resource to researchers world-wide and contribute to the advancement of medical research. Our goal is to eventually obtain mutant cell lines for every human gene." He continued: "At Haplogen we use this collection as an indispensable component in our research and

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development of antiviral medications for treating a wide range of harmful infectious diseases. This partnership of academic research at CeMM and private investment by the company has made it possible to finance this collection and build a resource to the benefit beyond the two institutions for scientists all over the world."

The cell line collection has received financial support also from ZIT (Technology agency of the City of Vienna) and can be explored at http://clones.haplogen.org

*Publication:

Bürckstümmer T, Banning C, Hainzl P, Schobesberger R, Kerzendorfer C, Pauler FM, Chen D, Them N, Schischlik F, Rebsamen M, Smida M, Fece de la Cruz F, Lapao A, Liszt M, Eizinger B, Guenzl PM, Blomen VA, Konopka T, Gapp B, Parapatics K, Maier B, Stöckl J, Fischl W, Salic S, Taba R, Knapp S, Bennett KL, Bock C, Colinge J, Kralovics R, Ammerer G, Casari G, Brummelkamp TR, Superti-Furga G & SM Nijman. Areversible gene trap collection empowers haploid genetics in human cells. Nat Methods. 2013 Aug 25. doi: doi:10.1038/nmeth.2609. [Epub ahead of print]

Haplogen GmbH is a privately held biotechnology company in Vienna, Austria, with active programs designed to combat viral infectious diseases. Haplogen employs an entirely novel genetics technology platform to identify novel host factors as candidate drug targets that block viral infection. This approach creates a higher barrier to the emergence of resistant virus strains, which is becoming a significant concern. Haplogen is building a pipeline of antiviral programs mainly in viral diseases that have no treatment option available.

Despite improved hygiene and successful vaccination programs, infectious diseases remain a major cause of morbidity and mortality. Emerging resistance to conventional drugs and a lack of scientific tools further add to this immense medical and socioeconomic problem.

Haplogen was founded in 2010 by the inventor of haploid genetic screens in human cell lines, Thijn Brummelkamp, together with scientists from CeMM, the Research Center for Molecular Medicine.

CeMM is an international, and interdisciplinary research Center in Molecular Medicine of the Austrian Academy of Sciences. "From the clinic to the clinic": Driven by medical needs, CeMM integrates basic research and clinical expertise to pursue innovative diagnostic and therapeutic approaches focused on cancer, inflammation and immune disorders. Located at the campus of the Medical University (MUV) and the Vienna General Hospital (AKH), Austria's largest medical research complex, CeMM functions as a bidirectional channel between basic research and clinical applications.

CeMM's innovative approaches are based on a novel, post-genomic, molecular understanding of biological and pathological systems. CeMM provides access to post-genomics technologies for the biomedical community and is a training and teaching center for a new generation of researchers in molecular medicine. The goal of CeMM is to assist in preparing the predictive, preventive and personalized medicine of the future.

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