XXIVth annual congress of the European Society of Gene & Cell Therapy (ESGCT) and

9th Stem Cell Clonality and Genome Stability retreat of the International Society for Stem Cell Research (ISSCR)

A travel award report by Alexandra Kuhn

This year´s annual conference of the European Society of Gene and Cell Therapy took place from the 18th to 21st October 2016 in the Congress and Exhibition Center in the beautiful city of Florence, Italy. For the first time, the ESGCT was enriched by the 9th meeting of the International Society for Stem Cell Research, taking place from the 17th to 18th October. Taking together numerous scientists and physicians from all over the world participated in this great event whereby 63 speakers were invited to give an oral presentation and 460 posters were presented under the title “Changing the face of modern medicine: Stem Cells and Gene Therapy”.

The ISSCR meeting started with a fantastic keynote speaker being nobody else than Keith Joung, giving an impressive presentation about defining, optimizing and customizing the specificity of CRISPR/Cas9 for genome editing. Besides the elegance and broad applicability of the RNA-guided Cas9 protein system, efficacy, safety and specificity are still major challenges researchers such as Keith Joung and his group are focused on. Referring to the urgent need of better detection methods for off-target events caused by nucleases, as they will repeatedly break their on- and off-targets until mutations occur (e.g. KO of tumor suppressors, activation of proto-oncogenes, oncogenic translocations), one important message of this talk was certainly to sharpen the awareness of *in silico* off-target analysis, which can only be one part of the final risk & safety analysis besides sequencing technologies. A genome wide, unbiased and highly sensitive analysis is the goal, but in fact *in silico* programs just consider specific parts instead of the whole genome. Finally, we have to do the best we can to develop good analysis tools to decrease the risk as much as possible for any therapeutic application, as no therapy is without any risk.

During the conference, I was particularly enjoying the presentations of Caroline Kuo, who gave a very well structured talk about targeted gene therapy in regards of X-linked Hyper-IgM syndrome, as well as the talk by Matthew Porteus highlighting the use of AAV6 for the delivery of CRISPR/Cas9 tools into HSPCs. An alternative to using allogeneic $CD34^+$ cells is the application of an autologous gene and cell therapy with e*x vivo* edited hematopoietic stem cells. As an overall effective and safe approach is still challenging, meanwhile, the group of Porteus has developed an elegant way to increase gene editing efficiencies in HSPCs for future therapeutic applications.

Another great presentation I wanted to point out was given by George Q Daley, who was addressing the challenges of directed differentiation of pluripotent stem cells into hematopoietic stem cells. He presented the progress of his team in “reprogramming” $CD34^+/CD45^+$ cells backwards to HSCs by the use of a certain gene cocktail.

Furthermore, the importance and progression of the gene therapy field was elucidated by introducing the first commercially available gene therapy product for the treatment of ADA-SCID, named Strimvelis. This *ex vivo* stem cell gene therapy is the first one of its kind operated by GlaxoSmithKline (GSK), Fondazione

Telethon (Telethon) and Ospedale San Raffaele (OSR) and was approved by the European Commission in 2016. Every single one of us is seeking for something to make the world a better place, why it is so important to achieve big breakthroughs from time to time and to keep up with our daily research.

Overall, this year´s ESGCT/ISSCR conference brought together an impressive number of high class researchers and their offspring listening to talks, poster presentations, making new contacts and enjoying the delicious Italian food at the Molecular Mingle Party in the Mercato Centrale, Florence.