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Precise and efficient editing of mammalian genomes for therapeutic purposes. How synthetic biology will impact therapies of the future?

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Marc Güell did his PhD in the Group of Dr. Serrano at Center for Genomic Regulation in Spain. In January 2011, he started as a Postdoctoral fellow at "Harvard Molecular Technology Group & Lipper Center for Computational Genetics" under the supervision of Dr. Church. From 2015 to 2016, he was a Wyss Technology Development Fellow at the Wyss Institute, Harvard University. Since spring 2017, Marc Güell is a Tenure track Professor of Synthetic Biology and principal investigator of the group "translational Synthetic Biology" at the Pompeu Fabra University, Barcelona.

Career advice:

Three key aspects for success in science are **creativity, impact and connection-to-society.**

• Think differently. Cultivate your imagination, basic science knowledge, inspiration from other disciplines (art, philosophy, design, …).
• Self-evaluate based in 'outcomes' and less in 'outputs' (number of citations vs number of publications; number of licenses/companies created vs number of patents; …).
• Extend yourself beyond own lab (collaborators, clinicians, industry, patient associations, social outreach, etc…).

Abstract:

One of the most advanced sequencing machines can sequence a human genome in hours but synthesizing ~30% of the yeast chromosome has taken years by a consortium of scientists. We are experiencing a growing gap between our capabilities to read and write DNA. This enormous sequencing capacity made the identification of alleles associated with biological processes easier than ever. However, the pace at which these alleles can be tested in the laboratory or addressed clinically has been limited. Advancement of gene synthesis and development of gene editing techniques increase to our ability to write DNA. Despite important progress, mammalian genome editing is still faces important challenges. Homology driven repair methods are still remarkably inefficient for most primary tissues or large edits, and integrative gene delivery methods such as transposases and lentiviruses cannot control insertion sites. Higher precision and efficiency in primary tissues or 'in vivo' genome editing is required to accelerate basic research and therapeutic translation.

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