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# Healx: Personalising Medicines for Rare Diseases

Silvia Sonzini

**Abstract:** Healx Ltd is a fast growing startup, founded in the Cambridge ecosystem by Dr. Tim Guilliams and his collaborators in 2014. The company focuses on the repurposing of known drugs for the treatment of rare diseases using a combination of machine learning algorithms, genomics and computational biology. Healx's focus is to find the right drug for the right patient, thus increasing the cost effectiveness and reducing drug wastage, as well as improving the patient benefit, which is fundamental to this social venture's mission. Healx is a pioneer in the field of personalised medicine and its developing technology has a real potential to change the quality of life for a vast number of people suffering from rare diseases.

**Keywords**: Healx, personalised medicine, rare diseases, machine learning, RarePurposing, Cambridge

## 1. The success story

Healx Ltd is a social venture, which focuses on the treatment of rare diseases in the novel field of personalised medicine. Rare diseases are a difficult market for big pharma companies, the number of patients is very limited and, in contrast, the cost for clinical trials is far too high to make the testing of new drugs in this field feasible. Nevertheless, some of the medicines that are already on the market for common diseases can be repurposed for treatment of rare diseases. The pinpoint is that these treatments only work for people with specific gene profiles.

Healx has put itself in the sweet spot, offering a platform that features big data technology and analytics across several databases owned by different organisations within the global life science and healthcare ecosystems; this system allows to efficiently match treatments to rare disease patients. Unfortunately, prescribed treatments do not work for every patient, which leads to poor patient outcomes and significant drug wastage. Healx's technology can help solve this problem by matching the right drug to the right patient.

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## 2. Where did it start?

While completing his PhD in Biophysical Neuroscience at the University of Cambridge, Dr. Tim Guilliams was actively involved in the field of science and technology. When he happened to read an online blog written by Matt Might [1], a parent entrepreneur in rare diseases, the seed for Healx started growing.

Matt's son was diagnosed with a congenital disorder named NGLY1, totally new to science and without a cure. Unfortunately, Matt's story is not an isolated case, there are 350 million people suffering from rare diseases worldwide [2].

Inspired by the challenges and determination of this family and many others, Tim decided to develop a platform enabling to source all the existing drugs and match them to the right rare disease. In order to do so, he approached three world experts in different fields: Dr. David Cavalla, Healx's Chief Scientific Officer, who worked for over 15 years in drug discovery and repurposing; Dr. Andreas Bender, Healx's Chief Technology Officer, the Reader in Molecular Informatics at Cambridge University; Dr. David Brown (Healx's Chairman), who has 40 years of experience within the pharmaceutical industry, is the co-inventor of Viagra, the world's most famous example of drug repurposing and the ex-Global Head of Drug Discovery at Roche. The team started to work on a possible treatment for Matt's son and in April 2014 Healx was rapidly established with the aim of helping rare disease patients find treatments for their disease.

## 3. Our technology

Currently, Healx's technology is based on repurposing known drugs for rare diseases. This is achieved through a combination of machine learning algorithms, genomics, and computational biology. The technology examines several different data types, such as genomic expressions and mutations, which allow the identification of drugs that may reverse the disease and result in a potential treatment. These candidate drugs are then tested and prepared for further clinical trials.

This technology is on the market under the name "RarePurposing". It is complemented by a tool named "RareOmics", which was developed by Richard Smith who joined the team in August 2015. RareOmics uses Natural Language Processing, enabling users to find and track the latest research and discoveries on their rare disease. In addition, Healx's technology has now been proven to also predict the response to drugs based on a patient's genomic profiling, which is incredibly exciting. This technology has the potential to increase the cost effectiveness and reduce drug wastage dramatically, as well as improve the patient benefit, which is fundamental to Healx's mission.

## 4. The journey so far

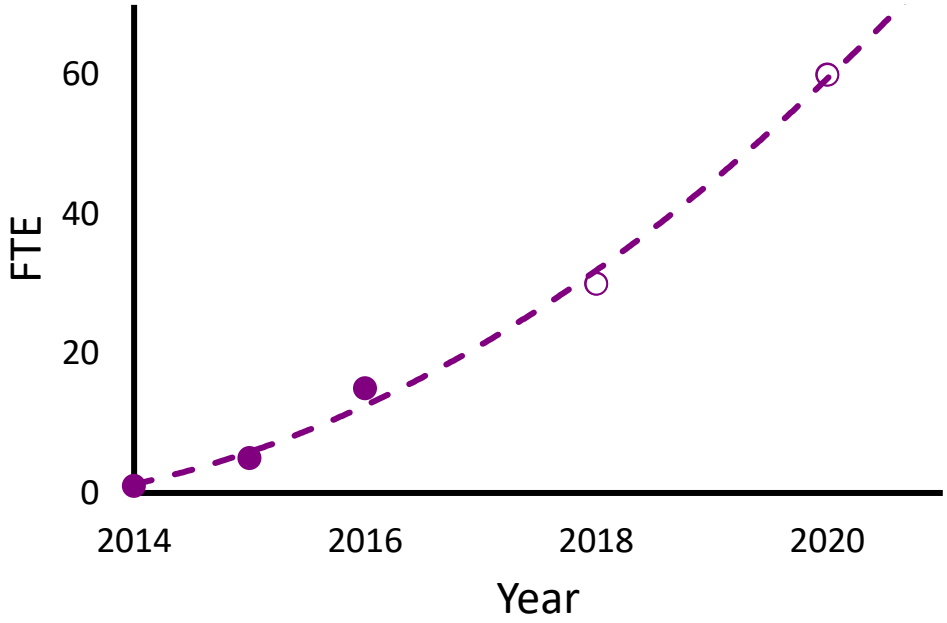


**Figure 1.** Full time employees (FTE) growth with foresight up to 2020. In 2014, Healx only counted 1 FTE, the number increased to 5 in 2015, and 15 in 2016. Due to the growth of the company, the number of FTE is expected to reach 30 in 2018 and 60 by 2020.

Healx's focus is to become a pioneer in the development of personalised medicine. The team has therefore developed a cloud-based platform that combines genomics, data mining and machine learning, which enables to find hidden connections between existing drugs and rare diseases.

Healx was born with a social mission focused on helping patient communities in rare diseases. The team is backed by the 'Blue Chip' investors from the Cambridge Cluster, including Dr. Hermann Hauser, Dr. Jonathan Milner and Dr. Darrin Disley [3]. The investors are fully aligned with Healx's mission and determined to help make a difference. To date, Healx raised over $2m and are preparing for a much larger funding round. Their journey started at Accelerate Cambridge [4], Judge Business School, a program that supports new ventures and allows them to get access to expert advice effectively.

Indeed, Healx has known a rapid growth and now counts a team of 15. They were awarded "Cambridge Graduate Business of the Year" 2016 and "Life Science Business of the Year" 2015 [5, 6]. They were covered in the Harvard Business Review about 'Transformative Business Models' [7] and were selected to meet with Her Majesty the Queen at the Royal Palace.

## 5. Looking to the future

As many know, the healthcare and drug discovery sectors are currently being disrupted by machine learning and artificial intelligence. Startups like Healx are pioneering this space and developing incredible value and hope for rare disease patients.

They have been one of the fastest growing startups of the Cambridge Cluster and are well on their way to become a leader in personalised medicine.

Healx is currently collaborating with companies both in the UK and USA, managing 10 projects and with 20 more in discussion for 2017.

A much larger funding round is on the horizon and fully personalized drug-matching models are currently being tested. This is an incredibly exciting space with the promise of potentially completely transforming the quality of life of millions of patients suffering from rare diseases worldwide. A startup and space to watch very closely.

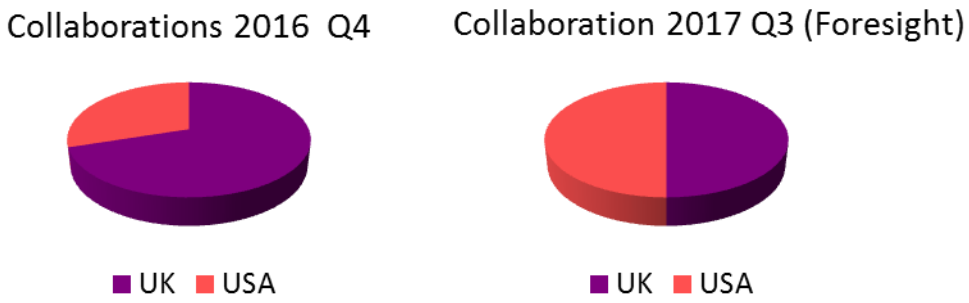


**Figure 2.** Project collaborations illustrated by country at the end of 2016 (left) and foresight for 2017 (right). At the end of 2016, 70% of the collaborations were established in the UK and the remaining in the USA; by October 2017, the collaborative efforts are expected to reach parity (50% each) between the UK and USA.

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## The company

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After obtaining her B.Sc. and M.Sc. in organic chemistry from Universita' degli Studi di Milano (Italy), **Silvia Sonzini** moved to England where she started a PhD at Melville Laboratory for Polymer Synthesis, within the Department of Chemistry, University of Cambridge. Silvia's research focused on non-covalent interactions between a family of macrocycles and aromatic amino acids within peptides and proteins of therapeutic interest, such as amyloid beta. Silvia then moved to MedImmune as a Postdoctoral Fellow within the Formulation Sciences team. In the last two years, she developed her scientific interest in antibodies and proteins engineering and formulation, aiming towards new drug delivery routes with particular focus on cancer and diabetes. Recently, Silvia joined AstraZeneca as Senior Scientist within the Advanced Drug Delivery team in the Pharmaceutical Science department. During her path, Silvia has always been interested in knowledge transfer between academic and industrial realities.