Dear all,

Findacure is all about making a big impact for patients with fundamental diseases. Our vision is to help create a major movement for patients that will transform their lives for the better.

As this report shows, we have already accomplished a significant amount since we became fully operational just over a year ago with the recruitment of Flóra Raffai as our first member of staff. We’ve implemented a range of innovative programmes, including training workshops, scientific conferences, and general awareness raising.

Feedback has been overwhelmingly positive. For instance, the representative of a patient group told us after one of our workshops:

‘I just wanted to say thank you for your advice at the last Findacure patient organisation workshop. Because of it, I found a UK group of researchers. They were very interested in our concept, as they are about to publish material that will support it. I found an appropriate grant to apply for as the proposal fitted all the criteria, the research chaps have sent off a proposal.’

This is what we exist for: providing hands-on, practical advice to patient groups in order to build their capacity to grow independently.

We have much more lined up for the coming year, with more patient workshops, a peer mentoring scheme and a first conference on drug repositioning: how to re-use generic drugs for rare diseases. This is something that will have a major impact on the prospects for patient groups to find treatments for their conditions, which is why we’re so excited about it.

Yours,

Nick Streeu
Findacure is a UK charity that is building the fundamental disease community to drive research and develop treatments. In undertaking this mission, Findacure follows in the footsteps of William Bateson, the father of modern genetics, who reminded us that we should ‘treasure our exceptions’.

**Mission:**

**Aims:**

1. Empower patient groups to become effective campaigners for change.
2. Facilitate patient groups to drive the development of treatments for fundamental diseases.
3. Campaigning for a receptive research environment that recognises the pivotal importance of fundamental diseases.
**Training Workshops:** Patient groups have an important role to play providing information to patients and their carers, as well as being partners in research with academics and pharmaceutical companies. We provide practical, hands-on training workshops for patient groups to provide them with the knowledge and skills to take on this important role. These workshops feature presentations from experts in the fundamental disease fields and case study examples from successful groups to inspire and educate the attendees.

**Peer Mentoring:** Fundamental disease patient groups are often set up by family members or a patient themselves. They transfer their own unique skills to the third sector and health sector with no previous experience in the fields. It can take years to become familiarised with information, processes, and best practice. To prevent each patient group having the ‘reinvent the wheel’, we run a peer mentoring scheme, matching up small or struggling groups with more experienced and established advocates to share their expertise and build a real sense of a fundamental disease community.

**Book Toolkit:** We are writing a book with a number of organisations and experts in the field of fundamental diseases to provide a practical guide to patient groups to help them tackle the issues they will face in developing treatments; from starting up a charity, to engaging with industry, to raising finance. This book should be published and available in 2015.

**Scientific Engagement:** Due to the rarity of fundamental diseases, they can be under-researched and under-funded compared to more common conditions. This results in a lack of understanding and treatments for millions of patients. To engage scientists and researchers around fundamental diseases and the importance of developing treatments for these neglected conditions, we organise annual scientific conferences.
Our proudest moments

This year has seen us go from strength to strength, and there’s a lot we’re proud of. Firstly, our Chairman Nick Sireau won ‘Inspirational Stakeholder of the Year’ in the 2014 ROAR Awards [Rare & Orphan Advocacy & Research Awards], and we were also nominated in the ‘Outstanding Patient Advocacy’ category. Indeed, awards were something of a theme of our year as we were also awarded the Bronze Award by Sanofi in their 2014 Patient Group Bursary Scheme Awards. For this, we were honoured at a reception hosted by the Baroness Neuberger DBE at the House of Lords.

Our crowdfunding campaign was a fantastic boost, and we ultimately raised $29,200 from 184 donations from 15 different countries. We were also awarded a grant from the BBC Children in Need fund, for the ‘Cross Your Own Oceans’ Project, which we will be running along with Elin Haf Davies.

Too, there was the success of our training workshops, which were attended by 124 people, representing over 62,550 patients in the UK and abroad. The feedback from these were very positive, with 93% of delegates saying it was relevant to their needs, 95% stating it increased their knowledge and skills and 100% saying the scheme was building a stronger, more connected fundamental disease community. This is hugely encouraging, and we have built further on this by launching a peer mentoring scheme. We have sixteen medical or charity experts who are going to share their experience to empower sixteen fundamental disease patient group representatives.

We became partnered with three rare diseases organisations; the RE(ACT) Community – a virtual place of meeting for rare disease stakeholders, international consortium INNORARE and Cures Within Reach, with whom we will be developing a drug repositioning programme for fundamental disease.

For the first time, we ran an essay competition for medical students to promote the necessity of studying fundamental diseases. The winner was Rosemary Grain, who our scientific board thought wrote “a thought provoking review” which kept them “intrigued from start to finish.”

Finally, our small team has grown – doubled in fact – with the addition of a second full time staff member. Louisa Ackermann started in early November, and is working as our Fundraising and Communications Officer.
My first Findacure workshop was by way of recommendation and I am so pleased that I took up the opportunity! I have learnt so much in a small space of time and put a number of strategies into practice almost immediately. I have since attended further workshops continue to develop my skills and knowledge of running a patient support group. I’ve learnt from others who have been successful in the rare disease field and benefitted from their experience. It’s helped me focus my energies into the things that matter and will actually make a difference in the short term.

- Allison Watson, Co-Founder, Ring20 Research & Support UK

Findacure is an invaluable help to small rare disease organisations such as The SMA Trust and SMA Europe. We need guidance to drive the development of treatments for Spinal Muscular Atrophy and Findacure offers just that. Findacure launched a Peer-mentoring Scheme to which I applied and I was very fortunate to have been selected. I now benefit from a monthly meeting with a clinical trials expert who is helping us navigate the difficult path to successful clinical trials.

- Vanessa Christie-Brown, SMA Europe Co-ordinator/ SMA Trust Research Co-ordinator.

“Findacure is an amazing organisation. If you have a rare disease, you’ll often feel like you’re a minority. But if you put all the rare diseases together, we’re actually the majority. The really interesting thing about Findacure is rather than terming them ‘rare diseases’, they’ve actually picked up that they’re fundamental disease. These diseases that are there with smaller patient groups, if you can get to the bottom of those and unlock the code, they give you so many clues about the more prolific diseases. So rather than thinking ‘let’s cure the big, sexy diseases that everybody knows about, unlock the codes to these tiny, smaller diseases, you will find out so much and get such value for money for the health system.”

- Pippa Palmer, Director of Active Patient Programmes, Active Futures

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“I am really keen to help Findacure in what they are trying to achieve. Rare diseases really struggle with recognition, but many of these diseases will go on to provide treatments for other conditions that are perhaps more common, and we can’t ignore them because people are suffering. We need to stop that now.”

- Charity officer at rare disease group for Birt-Hogg-Dubé Syndrome, the Myrovlytis Trust

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“For a small organisation like us, accessing expertise and building on the previous experience of other organisations who have gone through the same journey is really useful. There are so many different rare diseases, that if we all carry on in isolation, we’re forever reinventing the wheel. So an organisation like this (Findacure) that shares that information is really useful and helpful to us all.”

- Founder of the George Pantziarka TP53 Trust

“Cavernoma Alliance UK’s (CAUK) relationship with Findacure began through their very helpful series of workshops aimed at encouraging small charities. However, the most useful aspect came with the Findacure’s 2014/15 mentor partnership. Through this, Dr Turner is providing advice and introducing CAUK to people who can help with specific aspects of the work.”

- Ian Stuart, Founder, Cavernoma Alliance UK
Future Goals

As we grow, we hope to continue our commitment for patient support by developing an online portal to expand training support for patient groups, and thus make much needed support more accessible globally. We will also be continuing with our training workshops, the first of which will be held on January 30th 2015 and we hope our peer mentoring scheme will really develop over the next year.

Using funding from Children in Need, we will launch our Cross Your Own Oceans project to support young people living with fundamental diseases. This will allow them to organise their own challenges and take on an adventure to build their belief in not being limited by their conditions.

We are also hoping to develop social impact bonds for drug repositioning, a scheme which could potentially save the NHS millions in rare disease care, and to create our own crowd-funding platform for funding patient group projects.
We couldn’t have had such a successful year without the support of a lot of wonderful people. Firstly, our trustees; Dr Tony Hall, Dr John Solly, Julie Wall, Andy Milligan and Edna Kissman. They were instrumental help in our crowdfunding campaign, particularly John who offered matched funding and significant support with strategic planning.

Our scientific advisory board - Dr Rob Forsyth, Dr Anil Mehta, Dr Farid Khan, Prof Jim Gallagher, Dr Andrew R. Tee, Prof Lakshminarayan R. Ranganath, Dr Nathalie Kayadjanian, Prof Jonathan Jarvis, Oliver Timmis, and the newest addition, Prof Sir Tom Blundell – helped to judge our medical essay competition and are generally a fantastic source of help.

We also had some truly valuable work from our volunteers, who we would like to thank wholeheartedly for giving up their time for us. Rhys Dore, our first intern; Pippa Palmer, for her help with strategic planning; our LSE research team; our LBS strategic planning team, and all at Cambridge Hubs for their help with financial sustainability of projects.

Thank you!
Special thanks to...

- Cambridge CAB
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- Hogan Lovells
- Elin Haf Davies
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- AKU Society
- BBC Children in Need
- Myrovlytis Trust
- Golden Bottle & Bulldog Trust
- Donors to crowdfunding campaign
- Fundacion Genzyme
- Sanofi
- Awards for All
- Venture Partnership Foundation
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