Dear All,

2016 has been a big year for Findacure, filled with new project launches and growth of existing programmes. The year saw the fourth anniversary of the charity’s registration and our fourth member of staff join the team.

Our biggest undertaking has been a proof of concept study investigating the feasibility of our rare disease drug repurposing social impact bond. Through four focus groups, we captured the personal experiences of people living with a rare disease and the burden lack of treatment can have on them and their families. Through health economic modelling, we showed the huge cost untreated rare diseases impart on the NHS, as well as demonstrating the potential savings that can be realised through repurposing generic drugs for these conditions. We are looking forward to the next steps of this project in 2017, in trying to establish the world’s first drug repurposing social impact bond with NHS England and social investors.

Alongside this, we have expanded our support to rare disease advocates, empowering them to become more effective partners in managing their conditions. We continued our training workshop series, delivering four new workshops to 118 advocates. 2016 also saw the completion of our peer mentoring pilot and the launch of a brand new round of mentoring. Not only that, we also launched an updated version of our online portal and a brand new webinar series to offer more online support to those advocates unable to travel to in-person events.

It never ceases to amaze me how much Findacure is able to achieve in a year, which is only possible with our outstanding team and the support of our excellent pro bono collaborators. I welcome you to look through this report, to reflect on how we have been working to ‘treasure our exceptions’ in 2016.

Yours,

Flóra Raffai

Meet the team

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Why do we do what we do?

Rare diseases are defined by the EU as conditions affecting fewer than 1 in 2,000 people. Our vision and our mission are in response to the chronic need for better support for rare disease patients and for more research into treatments.

- In the UK, approximately **3.5 million** people live with rare diseases, which can be chronic, life-threatening, and isolating.
- The average rare disease patient waits **4 years** before they receive their final diagnosis, during which time they consult with **5 doctors** and receive **3 misdiagnoses**.
- Of the **7,000** recognised rare diseases, only **400** have licensed treatments.
- **2 in 3** patients and carers struggle to hold paid employment.
- **30%** of rare disease patients will die before their 5th birthday.
- Similarly, **3 in 5** patients felt their rare disease affected their education.
Our Aims and Work

Aim 1: Empowering Patient Groups

Our first aim is to empower patient groups to build their patient communities, develop as a charity, and drive treatment development.

Despite all the issues facing the 3.5 million rare disease patients in the UK, only half of them have a support group to turn to. Those that do exist tend to be ‘kitchen-table organisations’, set up by rare disease patients and advocates who often lack the skills, knowledge and confidence to get the group off the ground.

But patient groups have a fundamental role to play in addressing patient needs and representing patients in research. They are best placed to reach out to patients, to collate and provide credible information about disease progression, and to fundraise for and assist clinical research. We therefore work to empower rare disease patients and advocates by providing them with the tools to set up and grow their own dynamic patient communities.

How do we do it?

Training workshops
Our workshops focus on a specific challenge patient groups often face and help them to work through it.

Training webinars
Our webinars introduce patient groups to specific topics without requiring the commitment to travel to an event.

Peer mentoring
Patient groups and advocates can learn so much from each other. We partner small or struggling groups with more advanced groups or experts for tailored advice.

Online portal
The courses and guides on our portal are there for patients and advocates to access at any time and from any place!

By developing the capacity of patient communities in this way, we are ultimately providing better support and improved health outcomes for thousands of patients across the country.
2016 work and outcomes

2016 was a fantastic year for Findacure’s patient group empowerment projects. In addition to continuing our workshops, we launched a new online portal, organised our first three webinars, and completed our peer mentoring pilot scheme. Take a look at the molecules for our key achievements!

Case in point: Peer mentoring pilot
Of our 16 mentoring pairs, five created or improved their patient support literature, including one who developed a new helpline. One pair produced a series of videos to support patients enrolling in a new clinical trial, while two pairs closely supported the development of new clinical guidelines for their conditions. One pair made plans to fund a new treatment centre in the UK, and another organised a country-wide road-trip to re-engage beneficiaries. These are only a few of the many achievements our mentees and mentors made, and we are very proud to have helped accelerate the growth of patient groups in this way.
Aim 2: Collaborations and Drug Repurposing

Our second aim is to promote collaboration between rare disease stakeholders to facilitate treatment development.

95% of rare diseases do not have a licensed treatment in the UK. We truly believe collaboration between patients, clinicians, the pharmaceutical industry, medical professionals, and other interest groups, is necessary for the rare disease community to progress and confront its most pressing challenges, not least in research.

We also know that rare disease research is fundamental to our understanding of human biology and medicine, and is often at the cutting edge of clinical development. This means research into and the development of new treatments for rare diseases will ultimately benefit us all.

How do we do it?

Annual conference
Our conferences introduce and discuss the latest advances in rare disease research.

Networking events
Our networking events bring together a range of stakeholders to discuss pertinent topics and build collaborations.

Student essay competition
Creating awareness of rare diseases among doctors of the future is the aim of our ‘Student Voice’ essay competition.

Drug repurposing
We are building our own collaboration to repurpose existing generic drugs to specific rare diseases. This is much cheaper and quicker than conventional drug discovery—ideal for the underfunded rare disease sector! A project of this scale requires patients, patient groups, clinicians, policy makers, researchers and the NHS to work together.

What is the RDDR SIB?

We intend to fund our ‘Rare Disease Drug Repurposing’ project (see ‘Drug repurposing’) using a ‘Social Impact Bond’ (SIB). A SIB is a social finance mechanism. In our case, it means using the NHS savings that a repurposed drug generates to reimburse the cost of its clinical trial. To find out more, go to: www.findacure.org.uk/drug-repurposing
2016 work and outcomes

Our collaborative and drug repurposing projects really took off in 2016. Our Rare Disease Day conference, networking events and student essay competition were all significantly bigger than last year. We also launched and progressed considerably in our drug repurposing social impact bond (RDDR SIB).

Case in point: Children’s Hyperinsulinism Charity

In addition to our open projects for rare disease patient groups, we also forge more direct and long-lasting collaborations. Parents from the Children’s Hyperinsulinism Charity (CHC) approached us to help with their fundraising and research into the rare disease congenital hyperinsulinism. As their lead clinician had already identified a promising drug to repurpose, we decided they were the perfect patient group for us to get involved with.

In March 2016 we ran a full crowdfunding campaign for the CHC. Not only did this raise over £6,400, it also generated a lot of public attention for the charity and gave them re-usable assets in press releases and videos. We also decided to incorporate their research into our RDDR SIB project. The health economic modelling was completed in March 2016. It showed congenital hyperinsulinism costs the NHS £4.5 million each year, and that a repurposed drug could save over £470,000 over five years. We are now planning the clinical phase of the trial.
Don’t just take our word for it

Meet Allison

When Allison’s son was diagnosed with ring chromosome 20 syndrome, there was no patient group for her to turn to for support and advice. r(20) syndrome is a rare chromosome abnormality associated with recurrent seizures or epilepsy, intellectual disability, and behavioural difficulties. It is caused by fusion of the two arms of chromosome 20, forming a ring.

Allison increasingly realised the need for an organisation to support families and patients. There is no treatment for the condition, meaning awareness and patient support can be invaluable in managing symptoms and improving quality of life.

Allison co-founded Ring20 Research and Support UK CIO with a colleague. Their aim was “to pull together and signpost families, patients and health professionals to the latest information on r(20) through a ‘one stop shop’”, and to engage in and help fund important research.

But as a fledging patient group, many challenges lay head of them. This is where Findacure came in. Allison was paired with someone from a more advanced patient group in our peer mentoring scheme. They submitted the organisation’s first successful grant application, established a strong social media presence and strategy, and gained first-hand experience in running a family conference.

Our workshops also helped the organisation to draw up a strategic plan, to develop their communications, and to run events for families and other stakeholders.

“Findacure provides a unique service to rare disease patient groups. Their events allow sharing of information to inform best practice in a way that is cost-effective (budget, time and resources) to all”
Achievements and Proudest Moments of 2016

January

Launch of our drug repurposing social impact bond project, which aims to bring affordable treatments to rare disease patients

February

We ran our ninth workshop, focused on ‘Navigating the Highly Specialised Technologies’, to help patient groups understand how medicines are approved

Our Cross Your Own Oceans project brought young rare disease patients together

We held our ‘Drug Repurposing for Rare Diseases’ conference, in celebration of Rare Disease Day

March

Orphanet Journal of Rare Diseases published our 2015 essay competition winner

We launched a joint crowdfunding campaign with the Children’s Hyperinsulinism Charity for drug repurposing research and patient support

Completion of the congenital hyperinsulinism health economic model showed this rare disease costs the NHS £4.5 million, and that a repurposed drug could save enough to pay for a clinical trial

Findacure supporters ran the Silverstone Half Marathon, raising over £1,500

Rick and Flóra both presented at the Orphan Drugs and Rare Diseases Global Congress

“Thanks to the whole Findacure team. The conference was an excellent day with terrific speakers and a collaborative feel to the whole event”

“Findacure is such a help. It means small patient groups don’t have to recreate the wheel. They can learn from others who have already gone through different steps”
April
Rick visited the Wolfram Syndrome clinic in Birmingham and ran a patient focus group to assess the need for our drug repurposing project.

May
'Rare Diseases Collaborations' networking evening enabled a range of those interested in rare diseases from the Cambridge area to meet and learn about each other.

We welcomed Mary Rose, our Events Officer, as the fourth member of our team.

June
Rick chaired a session at the European Conference on Rare Diseases in Edinburgh.

We held a workshop on 'How Rare Disease Patient Groups Can Work With Researchers', to improve confidence and share best practice.

We filmed and edited the crowdfunding campaign video for aPod, a charity researching rare childhood cancers.

'Sustainable Development for Rare Disease Charities' workshop was held in London.

Flóra presented at Canada Talks Pharma in Montreal, on the topic of patient engagement in research.

Our first ever sky dive, featuring nine Findacure supporters, raised over £3,200!

“The workshops are relevant, interesting, informative and useful. The staff are friendly and helpful, and it is a good opportunity to meet others and network.”

“It was my first Findacure meeting and I found it very well organised. I met very interesting people and really enjoyed the networking evening. Thank you for running these events!”

“I like meeting people in the same situation. You don’t feel alone”
“I have the highest regard for what Findacure does. The rare disease world would be less without you!”

Our health economic modelling showed Friedreich’s ataxia costs the NHS £7.5 million a year. A repurposed drug could save enough to cover the cost of its own clinical trial.

Flóra was interviewed for BBC Radio Cambridgeshire about Findacure’s work.

July

Completion of the Wolfram syndrome economic modelling for our RDDR SIB. This rare disease costs the NHS £1 million, but a repurposed drug could save enough to pay for its own clinical trial.

August

We met with senior members of the NHS to present the proof of concept report for the drug repurposing social impact bond.

We had a fantastic night at our first ever fundraising gala dinner in Cambridge, in celebration of our fourth birthday.

September

Launch of our new online resources portal for rare disease patient groups.

2016-2017 Peer Mentoring programme launch event saw many of our 18 pairs meet for the first time.

“Not only did having a mentor mean that we achieved physical goals for the charity, it also helped us to gain confidence in our abilities and therefore more able to proceed with our aims”

“It’s a fantastic resource. Costing me a few hours of my time on a Friday afternoon, and what price is that to pay to find information I don’t believe I could easily find elsewhere?”
We launched our third ‘Student Voice’ essay competition for biology and medical undergraduates.

Findacure celebrated the fourth anniversary since registration as a charity.

The Midlands Rare Disease Showcase, organised by ourselves and Birmingham Children’s Hospital, welcomed over 80 rare disease stakeholders to network and learn about latest progress and to network.

Flóra presented, held a roundtable, and chaired a session at the World Orphan Drug Congress.

Our first Empowerment Advisory Committee was set up.

The Mill Cambridge’s weekly pub quiz was held in aid of Findacure.

We updated our website with new information and resources.

October

Rick spoke at both the Wolfram Syndrome UK and Ataxia UK annual conferences about our drug repurposing project.

The Royal Parks Half Marathon was completed by three Findacure supporters, who raised over £2,500.

“Findacure has helped enormously. They give you confidence and contacts, and a feeling that at least you know where to start.”

Patient group websites are vital points of contact and information for patients, but they can be difficult to create on a tight budget. Our webinar gave top tips from experts.

“I have made friendships with other patient group representatives. I very much feel there is a “Findacure family” atmosphere.”

November

Health literacy is important to consider when patient groups communicate with their patients. We held a webinar to highlight this and show best practice.

December

Flóra presented, held a roundtable, and chaired a session at the World Orphan Drug Congress.

The Royal Parks Half Marathon was completed by three Findacure supporters, who raised over £2,500.

The Mill Cambridge’s weekly pub quiz was held in aid of Findacure.

We updated our website with new information and resources.
Don’t just take our word for it

Meet Polly

When Polly was diagnosed with Mal de Debarquement Syndrome (MdDS) in 2006, very little was known about this condition. Meaning ‘bad disembarkation’, MdDS is a neuro-vestibular condition that may have a genetic component. It is usually triggered by exposure to motion, most commonly after travel, and is typically characterised by a rocking and swaying sensation, general ataxia, and impaired cognition. It is frequently misdiagnosed and misunderstood.

Due to recent research, Polly realized a defined need for a UK-based MdDS support group to represent patients and facilitate treatment development. Polly founded Action for MdDS UK with a friend in 2015. Her aims were to help members navigate the NHS to get an accurate diagnosis and to improve access to appropriate treatments.

Action for MdDS has already made excellent progress. They have made one of two trialled treatments available in the UK and are working with international researchers to develop these treatments and undertake further research.

Polly credits Findacure for providing her with the necessary knowledge, guidance and confidence to lead a support group and achieve these goals. After taking part in our peer mentoring programme, she felt able to “approach and respond to clinicians and researchers as an empowered patient with skills and information that can be useful to them”.

Our workshops were also “great opportunities for networking and discovering what other groups have been able to do successfully—and where they’ve run into difficulties and what they’ve learnt from these”. She says they have helped keep her motivated and determined to do more.

"Being part of Findacure’s peer mentoring programme was one of the best things I’ve done since diagnosis. Having a mentor gave our group credibility and helped me to regain a lot of lost confidence"
Thank You
We would like to thank the following people, groups and organisations for their support throughout 2016:

Volunteers
Rhiannon Stephens
Jane Scanlon
Zoe Letellier
Chris Rosser
Angela Wipperman
Samiksha Pattanaik
Jack Gawthrop
Lizzie Perdeaux
Julian Sireau
Pippa Palmer
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Andrea West
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Pip Hare
Lowri Morgan
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Nikki Curwen
Jade Toulson
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Rhia Toulson
Sammy Starr
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Katrina Bell
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Margaret Ogden
Lakshminarayan Ranganath
Kamlesh Sheth
Oliver Timmis
Andrew Tee
Prof Sir Tom Blundell
Jonathan Jarvis
Jim Gallagher
Natalie Kayadjian
Rob Forsyth
Anil Mehta
Farid Khan
Dear All,

As much as we have achieved in 2016, 2017 looks like it will be an even bigger and better year for Findacure. Our focus will be to establish ourselves as a truly UK charity, expanding our online empowerment projects and creating a roadshow of networking events travelling to Cardiff and Newcastle (with plans to move further afield in the following years).

We will look to create ever more opportunities for breaking down silos and encouraging peer learning with plans for our biggest ever scientific conference, full-day training workshops, and peer mentoring meet ups.

We will progress our rare disease drug repurposing social impact bond project to its implementation phase. We will continue to work closely with NHS England to set up the framework to implement the Rare Disease Drug Repurposing Social Impact Bond (RDDR SIB) alongside collaborating with financial and legal partners to create a viable investment vehicle.

We have an exciting year ahead of us: continuing to build a stronger patient voice, facilitating more research collaborations, raising further awareness of the importance of rare diseases. We welcome you to join us in our journey.

Yours,

Nick Simm

Letter from our Chairman