Dear All,

It has been a fantastic year for Findacure, with the charity going from strength to strength. Our team has grown to three full-time members of staff and several ongoing volunteers, which has greatly increased our impact.

2015 has seen the launch of three new empowerment projects: a year-long peer mentoring pilot, an online resource portal, and Cross Your Own Oceans. Our peer mentoring pilot saw 16 small patient groups linked with established patient communities and expert mentors. Over the past year, these groups have made real progress; with five groups registering as charities, ten groups developing their patient support services, and twelve increasing their links with research. In the words of one mentee, “I am passionate that smaller patient groups ‘get a voice’ through Findacure – this is where the organisation is at its best.” This is a primary aim of the charity, to build the confidence and skills of all people affected by rare diseases.

We have also launched our new drug repurposing programme this past year; looking at getting safe, effective, and cheap treatments available for patients. We’ve had Dr Rick Thompson join us, who has developed the social impact bond model and made the first steps towards partnering with the NHS. This programme will grow in 2016 with proof of concept models being built for the first three conditions selected for support.

We have big ambitions to build on this progress into 2016, with aims to run more training workshops, develop the online portal, and get plans underway for repurposing clinical trials. All of this to ensure advocates not only ‘get a voice’, but can use it to achieve the best outcomes for patients in the UK and beyond.

Yours,

Nick Senior
Co-Founder and Chairman
Our Vision
A world in which a vibrant rare disease community is collaborating towards treatments and cures

Our Mission
Findacure is a UK charity building the rare disease community to drive research and develop treatments

Our Team

Flóra Raffai
Executive Director
flora@findacure.org.uk

Libbie Read
Fundraising & Communications Officer
libbie@findacure.org.uk

Rick Thompson
Scientific Officer
rick@findacure.org.uk
1. Empower Patient Groups

Patients can be isolated from other patients, from healthcare professionals, from specialist centres, and from researchers. Support can be given by patient groups — yet only 50% of rare diseases have a specific group established, and they tend to be set up by patients themselves, who can lack knowledge of the third sector and healthcare sector.

Our programmes aim to bring together isolated patients, so they can take control of their diseases and meet the challenges of medical research and drug development. We run a series of training workshops, pair advocates with expert mentors to build the capacity of their organisations, and are building an online portal to provide patients and advocates with the tools and knowledge to become effective actors for change in their overlooked conditions.
2. Facilitate Treatment Development

Due to low population numbers, rare disease research and development is often viewed as being too costly by investors.

Of the 7,000 recognised rare diseases, only 200 have licensed treatments

Repurposing research takes drugs that are approved for the treatment of one disease, and tests their effectiveness in treating something different. We will commission clinical trials into these diseases with the aim of producing new treatments for diseases that would otherwise be neglected. Any successful trials will save the NHS money and improve patient health.

Findacure is developing a portfolio of drug repurposing projects, using an innovative social impact bond

"Findacure is my top network of 2015. An inspiring Findacure event was where we met the folks we are now collaborating with for a European Commission Horizon 2020 research grant."

We also encourage patient groups to launch their own research projects and clinical trials.
3. Campaign for a more Receptive Research Environment

Patients and advocates, researchers, clinicians, healthcare professionals, academics, biotech companies, and the pharmaceutical industry are all key stakeholders — but these groups are largely disparate and their interests can conflict.

By welcoming all stakeholders, we have consistently achieved a diverse audience. Alongside this, we organise an annual essay competition to raise awareness of rare diseases amongst the next generation of clinicians and researchers. We firmly believe that this inclusive and collaborative approach is fundamental to generating positive health outcomes for rare disease patients.

“I would like to underline the value of bringing together representatives of so many different groups; Findacure offers such a rare opportunity in this regard.”
Meet Maria

Maria has the rare disease Paroxysmal Nocturnal Haemoglobinuria (PNH), a rare blood disorder in which blood cells are produced without their usual protein sheath. This means that the blood cells are recognised as foreign bodies and are attacked by the immune system. She was diagnosed in 1997, but only met another patient with PNH a few years ago.

“I established PNH Support this year, attending Findacure’s workshops have helped upskill me in areas which I knew very little about before. I have set up Facebook pages/groups, a website, and a Twitter account - the messages which I try to disseminate on these platforms has been guided by the ‘Communications & PR’ workshop.

“Findacure is the only entity I know which benefits the rare disease community in such a way; it’s a brilliant resource.”

The fact that the workshops usually have patient group case studies is very useful. Learning from other patient groups who also attend these workshops about things that did and didn’t work for them has been helpful too. Best practice is always good to provide confidence.”

“I thought the workshops were very well organised and well attended. They have definitely ‘upskilled’ me!”
Achievements and Proudest Moments of 2015

January

Nick spoke at BIG Data in Pharma Europe Conference on 'Social Media for Patient Recruitment'

February

We held our Scientific Conference on Drug Repurposing

Nick spoke at Pharma Stream webinar on 'Supply Chain Excellence'

March

Our Cross Your Own Oceans project, the first to specifically target youths, was launched

"Findacure has helped me move from being a patient on disability leave to someone who has the tools to stand up and make a difference."

"My two colleagues and I were inspired by the stories of others, who had been through the journey and emerged with really impressive achievements."

"I gave full marks for an excellent event, which gathered practitioners in the art of drug repurposing for rare diseases with a diverse audience. This produced useful discussions, particularly in the breaks."

"It was my first Findacure meeting and I found it very well organised. I met very interesting people and really enjoyed the evening."
May

Eleven Findacure supporters took part in the Superhero Run, raising over £1,700.

“As individuals, we find it very difficult to speak with a sufficiently loud voice to be heard. Bringing together the critical mass needed to speak loudly with a common voice is incredibly important.”

June

We ran our seventh patient workshop, on working with the pharmaceutical industry.

Flóra and Nick were each shortlisted for Tribute to Champions of Hope Award for Collaborations in Advocacy

Flóra chaired a panel session, ‘From Patient to Progress’, at the eyeforpharma Patient Summit

April

We ran our sixth patient training workshop, focussed on communications and PR

Day Three of Cross Your Own Oceans

Day Two of Cross Your Own Oceans

97% of all workshop attendees believe it is useful for rare disease patient groups to share experiences

Rick, our Scientific Officer, joined the team

“When you work in a small patient organisation, it can be quite isolating. It is good to have an outside voice to bounce ideas off.”
Our eighth training workshop on Crowdfunding took place.

Costello Medical Consulting helped us, pro bono, to model the cost of CHI for a drug repurposing project.

The Cambridge Rare Disease Network (CRDN) Conference was co-organised by Findacure.

Libbie, our Fundraising and Communications Officer, joined.

100% of network evening attendees found it worthwhile, relevant and interesting.

83% of users believe our online training portal meets an important unmet need.

"Having a mentor has helped me turn a corner from feeling cornered.”

July

Sony Mobile Challenge helped us, pro bono, to develop a communications strategy.

August

Two boats competed in the Rolex Fastnet Race in aid of Findacure, raising over £3,400! Thanks to Elin Haf Davies and Chris Frost, and Flic Gabby and her Elixir crew.

Flóra chaired a session at the British Science Festival and spoke at the Orphan Drug Summit in Copenhagen.

September

Nick ran a masterclass on patient collaboration with industry at the Global Orphan Drugs and Rare Diseases Congress.

We held our first networking event, which brought together a range of rare disease stakeholders.

Costello Medical Consulting helped us, pro bono, to model the cost of CHI for a drug repurposing project.

The Cambridge Rare Disease Network (CRDN) Conference was co-organised by Findacure.

“Having a mentor has helped me turn a corner from feeling cornered.”

83% of users believe our online training portal meets an important unmet need.
Twenty Findacure runners completed the Royal Parks Foundation Half Marathon, raising over £7,200.

Flóra was interviewed on BBC Radio Cambridgeshire about our Cross Your Own Oceans Project.

Rick spoke about ‘Economic Impact of Rare Diseases’ at the Health Economic Global Congress.

The Big Give raised almost £5,000 for our patient empowerment programmes.

Nick chaired Day One of SMi Orphan Drugs and Rare Diseases Conference.

Flóra spoke on the ‘Creating and Protecting a Patient-Centric Value Strategy panel at the Pharma Integrates conference.

Five Findacure supporters donned their red jackets and hats for the Victoria Park Santa Run, raising over £700.

“I have experienced so much personal development in my own role, and have recieved a huge amount of information about supporting families and members.”

87% of attendees believe the workshops increased their knowledge and skills.

“We’re a reasonably well developed patient group, but we do not have the expertise, knowledge or skills needed to deal with the interest from scientists in Niemann Pick. Findacure offers us great help and guidance.”

“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.”
Don’t just take our word for it

Meet David

David attended three of Findacure’s workshops in 2015. He is a member of two rare disease patient support groups: Turner Syndrome and Ectodermal Dysplasia. Neither of these two conditions have specific treatments available, there is only symptomatic care for patients. He got involved in these groups through his past professional roles in the pharma industry.

“I think my first meeting was focussed on identification of patients with rare diseases. This is central to much that I do, so highly relevant. I have found all other workshops equally relevant and content practically oriented. I always learn new things and I share what is relevant with the two patient groups."

“My confidence in advising and supporting groups has increased. Working in relative isolation always gives cause for doubt that something significant has been overlooked.”

“I have found all the workshops relevant and practically orientated.”

I would like to underline the value of bringing together so many different groups; Findacure offers such a rare opportunity in this regard. Although I don’t manage a group, my confidence in advising and supporting my groups has increased.”
Goals for 2016

Findacure’s strategic plan includes five areas of focus for 2016

1. Establish a strong foundation for patient group empowerment projects

To create a replicable model for all empowerment projects, that can be built on in the future.

2. Develop proof of concept for drug repurposing and social impact bond

Test non-profit drug repurposing and the social impact bond as viable models for accelerating therapy development.

3. Build our scientific outreach programmes

To improve collaboration between diverse stakeholders, and to establish Findacure’s reputation as a key player in the rare disease research field.

4. Diversify and grow the charity’s income

Increase our income from a combination of trusts, corporate partnerships, community fundraising, and events.

5. Expand Findacure as an organisation

Increase staff numbers and recruit volunteers to build the capacity of the charity to meet the needs of our beneficiaries.
Thank you

We would like to thank the following people, groups and organisations for their

**Advisors**
- Dr Nick Sireau
- Dr Tony Hall
- Dr John Solly
- Julie Walters
- Edna Kissman
- Andy Milligan
- Dr Rob Forsyth
- Dr Anil Mehta
- Dr Farid Khan
- Prof Sir Tom Blundell
- Prof Jim Gallagher
- Dr Bruce Bloom
- Dr Nathalie Kayadjian
- Dr Andrew Tee
- Prof Lakshminarayan Ranganath
- Prof Jonathan Jarvis
- Oliver Timmis

**Fundraisers**
- Flic Gabbay and Elixir Crew
- Elin Haf Davies and Chris Frost
- Superhero Runners
- Royal Parks Half Runners
- Santa Runners
- Myrolytis Trust
- Rotary Club of Ely Ely Hereward Rotary Club
- Stone King LLP
- Morrison’s Foundation
- Wasps Legends Foundation
- Napp Pharmaceuticals
- Venture Partnership Foundation
- BBC Children in Need
- Big Lottery Fund
- Genzyme
- Labstract
- Congenica
- Costello Medical Consulting
- TranScrip
- Linguamatics
- Candis Magazine
- Stamp collectors
- Big Give donors

**Volunteers**
- Emma Finnerty
- Rhiannon Stephens
- Zoe Letellier

**Pro Bono**
- White & Case LLP
- Oliver Wyman
- Sony Mobile
- Costello Medical Consulting
- Hogan Lovells LLP
- Elsevier
- Women Hack for Non-Profits
- Numbers for Good
- WorkSpace
- Diorama Studio
- Speakers at events
- Peer mentoring mentors
I love findacure because... 
they bring the Rare Disease Community Together "like a family"

I love findacure because... their work changes so many different lives...

I love findacure because... they bring patients, scientists and doctors together!
Treasure Your Exceptions

www.findacure.org.uk