Dear All,

The past twelve months have seen some great successes for Findacure, including our biggest conference to date. Our Rare Disease Showcase series continued to grow, with three events in Cardiff, Cambridge, and Newcastle helping to build rare disease networks across the UK. Our first full webinar programme was completed in 2017, providing patient groups with readily accessible training on six wide ranging topics and we have also implemented a new full-day format for our workshops in response to your feedback. We have created more opportunities for patient groups to share their experiences, and developed interactive sessions during the day. This change has been very well received, with more attendees than ever before reporting an increase in their knowledge and skills.

2017 was also a year of change for the charity, with our Executive Director Flóra Raffai moving to pastures new. Since becoming Findacure’s first employee in 2013, Flóra worked tirelessly to grow the charity, developing all of our programmes, and becoming an expert in rare disease patient advocacy. Flóra is a very hard act to follow, but it has been my privilege to lead the charity since May 2017, and carry on her excellent work. I’m truly excited about our plans for the future, with an evolving website, an expanded e-learning platform, and ever increasing opportunities for patients to engage with industry, researchers, and one another.

I am immensely proud of our achievements in 2017, and exceptionally grateful to all of our supporters, sponsors, and volunteers who help us deliver our work year on year. Most of all though I would like to thank the patient groups that we work with. Their continued dedication to their patients and desire to deliver change in the field is inspiring and a constant motivation for all of our work.

Yours,

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Rare diseases are defined by the EU as conditions affecting fewer than 1 in 2,000 people. Due to the complex symptoms and low patient population of each rare disease, living with one can be one of the most painful, devastating, and isolating experiences imaginable. Findacure wants to change that.

Why do we do what we do?

Rare diseases are defined by the EU as conditions affecting fewer than 1 in 2,000 people. Due to the complex symptoms and low patient population of each rare disease, living with one can be one of the most painful, devastating, and isolating experiences imaginable. Findacure wants to change that.

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.

Similarly, 3 in 5 patients felt their rare disease affected their education.

30% of rare disease patients will die before their 5th birthday.

75% of rare diseases affect children.

Disease-specific patient groups are a crucial source of reliable information and support to patients, but can also have a huge impact on research and policy. Findacure’s central purpose is to help these patient groups to fulfil their potential and transform their field. We provide training to help these groups and patients feel positive, confident, and empowered to make change. We are building a rare disease community with the strength to support their needs.
Our aims and work

Aim 1: Empowering patient groups

Patient groups provide essential patient support and drive research forward. Despite this, only around half of all rare conditions have a patient organisation to represent them.

Existing groups tend to be set up by rare disease patients and family members who, although greatly motivated, often lack the skills, knowledge, and confidence to get the group off the ground. 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.

Findacure aims to support patient groups in four major areas:

- Building their group into a fully registered and professional charity
- Getting involved in research to deliver new treatments to patients
- Supporting patients and families to live with a rare condition
- Raising awareness among clinicians and the public about their condition

How do we do it?

Workshops
Our day-long workshops focus on a specific challenge patient groups often face and help them to work through it alongside other patient groups.

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.

E-learning 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.
2017 work and outcomes

2017 was a fantastic year for Findacure’s patient group empowerment projects. We completed our first full webinar programme, addressing topics such as health literacy and medical research, and continued to grow our online portal with new courses on building your team, health technology and everything in between.

Case in point: Peer mentoring programme

Our 2016-2017 cohort had great success, with all 16 of our mentoring pairs making significant progress towards their goals, ranging from raising awareness to progressing research and treatments. Four patient groups registered as new charities, three implemented new branding, two launched new websites and one successfully achieved medicine reimbursement for a subset of patients. These are just a few of the many achievements our mentees and mentors made and we are thrilled to have helped accelerate the growth of patient groups in this way. We received overwhelmingly positive feedback, with one mentee telling us: ‘It has had a hugely positive effect on my patient group...I did not know where to start’.
**Aim 2: Promoting collaboration**

95% of rare diseases do not have a licensed treatment. We believe collaboration is the key to progress in research.

Findacure aims to promote collaboration between rare disease stakeholders to facilitate treatment development. We truly believe collaboration between all 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**
A chance to discuss the latest advances in research and unite the rare disease community.

**Showcases**
Held across the UK, these events highlight excellent rare disease projects and allow patients, industry, and academia to network and build collaborations.

**The Student Voice essay competition**
This raises vital awareness of rare diseases among doctors of the future, with the winner published in Orphanet Journal of Rare Diseases.

**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.

**Case in point: Lightning talks**
This year’s conference and Showcases featured 24 lightning talks delivered by our event delegates. These short five minute talks give attendees a chance to take to the stage and share their own work with the wider community. Nine of these talks were given by patients or patient organisations in 2017, giving them the chance to share their stories with researchers, biotechs and industry.
2017 work and outcomes

Our collaborative projects grew even stronger in 2017, including our biggest conference to date. We reached rare disease advocates throughout the community, helping to build rare disease networks across the UK with our three Showcases and engaging students internationally with our essay competition.

Case in point: Drug repurposing open call

From March to May 2017, we asked people around the world to share their drug repurposing ideas for rare diseases, with the aim of advancing the best of these projects, either through support or the identification of funding opportunities. We received 38 promising project ideas, demonstrating the great potential of patient group-led innovation in this area. Of the proposed projects...

- 20 are US based
- 5 are UK based
- 4 are based in Europe
  - 1 Canadian, 1 Australian
  - 1 South African
- Of those beyond the UK
  - 21 have a UK collaborator

- 3 preclinical studies
- 6 phase I studies
- 24 phase II or III studies
- 17 proposals include patient group collaboration
- 29 target rare genetic conditions
- 12 conditions have no treatment

Over 120 people attended the conference, including academics, clinicians, patients and representatives from charities, biotech and pharma

Our Drug Repurposing Conference was held on 28th February to celebrate Rare Disease Day

The winning essay has been viewed online on OJRD over 3000 times

Our essay competition received entries from students across the world, including Asia, Australia, North America and Europe

We held three Showcase events, in Cardiff, Cambridge and Newcastle

Over 147 people attended our Showcase events

Our Showcases included 30 inspiring talks from people across the rare disease community
Don’t just take our word for it

Meet Wendy

When Wendy’s son was born with Norrie disease, a rare genetic condition that causes blindness, hearing loss, and developmental delays, there was no organisation for her to turn to for support and information. Determined to help others herself, but in need of guidance on how, she joined our peer mentoring programme in September 2016 and was paired with Kamlesh, who runs a pharma-focused consultancy.

In less than nine months, Wendy secured full charity status, set up a new scientific advisory panel, and ran a launch event for the charity, to name just a few achievements. After being told there were only six UK families affected by Norrie disease, she has now made contact with over 30.

“Kamlesh's patience has been incredible and the NDF are so grateful to him, and of course Findacure, for making this amazing opportunity happen. I certainly don't think the NDF would be where we are now without Kamlesh’s input for this year.”

After completing the peer mentoring programme Wendy and the NDF have gone from strength to strength. They now have a PhD student researching hearing loss in Norrie disease at University College London, and are considering a Norrie disease registry as an option for the future. Wendy is also acting as a mentor for another new patient group in Findacure’s 2018 mentoring programme.

The Findacure team are exceptionally proud of all of the achievements of our peer mentees, but the progress made by Wendy and the NDF is a tribute to their commitment and dedication. It hopefully shows what can be achieved by any patient or parent with a little guidance and a lot of hard work.
Achievements and Proudest Moments of 2017

January

The winners of our 2016 Student Voice essay competition were announced, with the winning essay published in the Orphanet Journal of Rare Diseases

February

We held our ‘Drug Repurposing for Rare Diseases’ conference, with over 120 delegates joining us to celebrate Rare Disease Day

March

Our firewalk saw brave fundraisers walk over hot coals to raise money for Findacure

After launching our Open Call, we received over 30 submissions of potential drug repurposing projects, highlighting the need for more attention in this research area for rare diseases

“I love Findacure because I always leave their events feeling inspired”

“It has been the most rewarding year on Findacure’s peer mentoring scheme, I cannot thank them enough”
We said goodbye to our beloved Executive Director, Flóra Raffai, and hello to Rick as our fantastic new CEO.

We partnered with Genetic Alliance to help early-stage patient groups in our ‘A-Z to Setting up a Patient Group’ workshop.

Our CEO, Rick, spoke at the Oxford Rare Disease Initiative Symposium.

We attended and exhibited at the ABPI annual conference.

Stuart Brien raised an incredible £3,000 for Findacure by running in the London Marathon.

Our Cardiff Rare Disease Showcase focused on the great work happening in South Wales for our first showcase of 2017.

‘Engaging your Community for Fundraising’ workshop was held in London.

Our fundraisers went above and beyond with Julie Walters completing the London to Cambridge cycle, Stuart Penny completing the Megavalanche and Tom Hancock completing the Three Peaks Challenge.

“Findacure’s workshops are particularly beneficial in getting conversations going across organisations and breaking down barriers to understand other people’s perspectives.”

“Thanks to Findacure, I will be more confident in pursuing my goals.”

“Findacure do a fantastic job, the conference was very well organised and enjoyable, I am so grateful for their hard work.”
We exhibited at the 2017 ON Helix conference.

In August, we updated and uploaded new courses to our e-learning portal on topics including health economics, patient registries and health technology assessment.

Our second sponsored Sky Dive saw seven daring supporters raise a brilliant £3,500 for Findacure.

Our ‘Working with Industry’ workshop helped patient groups understand how they can build successful collaborations.

We ran our Cambridge Rare Disease Showcase for the third consecutive year to bring together the city’s vast rare disease community.

We welcomed Katie, our Fundraising Officer, as the newest member of the team.

Rick gave a talk at Oxford Pharmagenesis.

We celebrated the success of all sixteen patient groups who graduated at our 2016-2017 Peer Mentoring closing event.

“Without a doubt, I always learn something new at Findacure events.”

“The networking opportunities that Findacure provides are invaluable, we can learn so much from each other.”

“The peer mentoring scheme and all the Findacure resources have been fantastic, without them we would not be here.”
We launched our fourth ‘Student Voice’ essay competition for biology and medical undergraduates.

Our final workshop of 2017 discussed the different ways patient groups can help patients gain ‘Access to Medicines’.

Our creative supporters donated beautiful handmade gifts to sell on our stall at Mill Road Winter Fair.

Rick spoke about drug repurposing at the World Orphan Drug Congress in Barcelona.

Cambridge Sing! choir helped us fundraise with festive carols at Cambridge train station.

Our Newcastle Rare Disease Showcase welcomed over 70 rare disease stakeholders to network and learn about the latest progress in the North East.

We partnered with Kudos Health on a workshop to prepare patient groups for the new GDPR.

Libbie took her dedication to another level when she completed a gruelling triathlon to raise money for Findacure.

The Royal Parks Half Marathon was completed by six wonderful Findacure supporters, raising over £3,000.

Rick spoke about ‘accelerating rare disease innovations to market’ at the inaugural Rare Innovations event.

“I love Findacure because they give rare disease patient groups a voice”
Don’t just take our word for it

Meet Diana

The Ectodermal Dysplasia Society has been providing vital help and information to patients since it was founded in 1996. As Founder and CEO, Diana was conscious of the lack of information available specifically for children with the condition. She joined Findacure’s 2016-2017 peer mentoring programme in the hope of learning how to best help young patients and was matched with Lizzie from Oxford PharmaGenesis.

Diana aimed to enable children to better understand terminology used by doctors and access accurate information on ED easily without always having to ask their parents. With Lizzie’s support, Diana successfully completed a survey to understand the needs of young patients, set up two ‘secret’ Facebook groups for different age groups and worked with a graphic designer to design an age-appropriate web app. These are just a few of Diana’s achievements and she has expressed immense gratitude for how Lizzie and the peer mentoring programme helped to keep her on track, improving the lives of young patients living with ED.

“Having a mentor has given me encouragement, provided me with ideas and a different way of thinking. It’s kept me focussed and without them, I would not have got this far.”

Once again, the progress made by Diana and the ED Society is a tribute to their admirable dedication and commitment. We are exceptionally proud of all our peer mentee graduates and Diana’s story is one of many that demonstrates the importance of collaboration and the power of patient groups.
Thank you

We would like to thank the following people, groups, and organisations for their generous support in 2017:

Volunteers
Callum Appleby
Duncan Batty
Vanessa Christie-Brown
Elinor Clark
Ellie Collins
Rebecca Cosgriff
Mark Edwards
Debra Fine
Tina Flatau
Flic Gabbay
Narissa Gipp
Josie Godfrey
Kate Hanman
Lesley Harrison
David Head
Sean Kelly
Julian Isla
Avril Kennan
Bence Kocsis
Yan Lai
Alexandra Latcham-Ford
Dan Lewi
Robyn Marshall
Nick Meade
Linda Morrissey
Polly Moyer
Sharmila Nikapota
Carol Nwosu
Andrew O’Brien
Busty Okundaye
Susan Ollier
Pippa Palmer
Kay Parkinson
Susan Passmore
Samiksha Pattanaik
John Pearson
Lizzie Perdeaux
Diana Perry
Jill Prawer
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Stefi Rucci
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Rebecca Starkie
Lila Stavropoulou
Janette Thomas
Patsy Thomlinson
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Oliver Timmis
Tania Tiraoro
Julie Vallortigara
Sarah Venugopal
Lindsay Weaver
Andrea West
Jeremy Wortzel
Tara-Lee York

Fundraisers
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Flóra Raffai
Libbie Read
Odette Read
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Mark Regan
Stephanie Riber
Chris Rosser
Sammie Starr
Rory Stobo
Amalia Thomas
Kate Thorogood
Emily Varley
Julie Walters
Cambridge Sing! Choir
Thaxted Flower Club

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Mark Edwards
Tony Hall
Edmund Jessop
Kathryn Johnson
Edna Kissman
Deborah Mann
Andy Milligan
Jon Morton
Margaret Ogden
Lakshminarayanan
Ranganath Kamlesh Sheth
Nick Sireau
John Solly
Alan Thomas
Julie Walters
Dear All,

Living with a chronic disease is hard. The relentlessness of illness can wear down even the most positive and tenacious of people. But living with a rare chronic disease is even more difficult. Countless visits to different doctors, misdiagnoses and a general lack of awareness make it a particularly isolating experience.

When my children were first diagnosed with the rare disease Alkaptonuria (AKU) 17 years ago, there was no support group to turn to. That’s why we teamed up with a patient to set up the AKU Society patient group. That changed everything: suddenly patients had a unified voice that could campaign on their behalf. Workshops brought patients and carers together, often for the first time, and patients successfully became involved in scientific and clinical research.

Findacure exists to replicate this on a much larger scale and for many more rare diseases. As this impact report shows, there has been significant progress over the past year in empowering rare disease patients and helping them grow their groups and advocate on their behalf. Supported by a tireless and hard-working team of staff and volunteers, Findacure has also promoted closer collaboration between patients, clinicians, academics and industry in order to develop new treatments. With only 400 out of 7,000 rare diseases having treatments, there is a crucial need to keep pushing in this area.

Thank you for your support.

Yours,

Nick Squire
Connect
Keep in touch with us via social media for updates on upcoming events, opportunities and the latest news in rare diseases. You can find us online using @findacure_fdn

Donate
We rely on the generosity of our supporters to provide us with the funds and power to build a stronger rare disease community. To donate, visit our giving page at www.findacure.org.uk/donate

Volunteer
We are always on the lookout for volunteers who can make a big difference to our work by offering their time and skills to support us. For more information, contact our Projects and Comms Officer via libbie@findacure.org.uk

Fundraise
From running marathons to singing carols, raising money for us provides support that makes our work with patient groups possible. To get involved, contact our Fundraising Officer via katie@findacure.org.uk

Collaborate
Build a partnership that allows your employees to use their expertise and deliver impact across the rare disease community. To discuss collaboration opportunities, contact our CEO via rick@findacure.org.uk

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www.findacure.org.uk