UK GENETIC DISORDERS LEADERSHIP SYMPOSIUM

Agenda

Friday 9 March

From 3.00pm onwards	**Registration**
7.00pm	**Welcome drinks** (1st Floor, The Regency)
8.00pm	**Welcome dinner** (1st Floor, The Ballroom)

Saturday 10 March

Time	Title	Speaker	Summary
8.45am	Welcome	**Caroline Harding** CEO Genetic Disorders UK	Caroline Harding will welcome delegates and provide an overview of the day's programme.
8.50am	New genetic therapies for rare diseases: from gene replacement to gene repair	**Professor Stephen Hart** Professor in Molecular Genetics UCL Great Ormond Street Institute of Child Health	Professor Stephen Hart will talk about the importance of recent advances in gene therapy, including editing of the genome to repair mutations from larger regions of DNA down to the level of single bases, as well as correcting single bases in RNA molecules.
9.20am	The future of life sciences after Brexit, and recent developments within specialised commissioning in England	**Ed McIntosh** Associate Director Incisive Health Specialised Healthcare Alliance	Ed McIntosh will review the UK government's first life sciences sector deal since Brexit and provide an update on developments within specialised commissioning in NHS England.
Accessing services, therapies and medicines when funding is scarce			
9.40am	Making the case for service funding to NHS England	**Fiona Copeland** Chair PCD Family Support Group	Fiona Copeland will speak about how a collaboration between clinicians and the PCD Family Support Group led to the funding of 'cradle to rocking chair' services for patients with primary ciliary dyskinesia in NHS England.
9.55am	NHS England's decision-making process: a charity's perspective	**Simon Butler** Head of Policy and Public Affairs Anthony Nolan	Simon Butler will share the charity's experience of engaging with NHS England's funding decision-making processes.

10.10am	Bridging the access gap	**Romina Oxborough** Director **Lucia von Bredow** Patient Advocacy Manager Clinigen Consulting	Romina Oxborough and Lucia von Bredow will talk about the routes that are available to patients when a medicine is not readily accessible.
10.30am to 11.00am		**BREAK**	

How information technology is revolutionising the genetic disorders world

11.00am	Democratising rare disease diagnosis	**Dr Peter Fish** Clinical Lead Mendelian	Dr Peter Fish will present Mendelian, the first online rare disease diagnosis service, which uses state-of-the art data science to streamline the diagnosis odyssey for families affected by a genetic disorder.
11.15am	Whole-genome sequencing: a universal test for genetic disorders?	**Neil Ward** Director of Marketing EMEA Illumina	Neil Ward will explain how improvements in technology and chemistry have led to significant advances in the cost and speed of whole-genome sequencing, and what is now made possible by this progress.
11.35am	Empower and Enable: The power of words	**Jason Gordon** Health Manager Texthelp	Jason Gordon will explore how assistive technology can break down barriers, helping to create inclusive organisations and accessible patient information.
11.50am	How to use social media to strengthen your message and grow your audience	**Sam Carlisle** **Sally Land** Co-founders Cause Communications	Sam Carlisle and Sally Land will provide an overview of the different social media channels and how a genetic disorder charity might use social media to engage with members and highlight key issues for their cause.
12.05pm	How match funding can boost your charity's online fundraising	**Laura Walmsley** Operations Executive The Big Give	Laura Walmsley will explain how match funding can help charities galvanise their fundraising and increase supporter engagement, and talk about the UK's largest online match-funding campaign, the Christmas Challenge.
12.25pm	In it to win it! Changing the future for children born with fibrodysplasia ossificans progressiva (FOP)	**Chris Bedford-Gay** Founder and Chair FOP Friends	Chris Bedford-Gay will talk about building a sustainable small charity supporting patients and families, and punching above your weight in the pursuit of your charity's ultimate goals.
12.40pm to 2.00pm		**LUNCH**	

2.00pm	Gene silencing in Huntington's disease – a step closer to disease-modifying treatments	**Dr Sarah Mason** John van Geest Centre for Brain Repair University of Cambridge	Dr Sarah Mason will discuss recent advances in the field of disease-modifying therapies in Huntington's disease and the implications that this research may have for other neurodegenerative genetic disorders which feature the build-up of toxic proteins in the brain.
2.30pm	Building a visible, well-funded genetic disorders community	**Caroline Harding** CEO Genetic Disorders UK	Caroline Harding will talk about the UK Genetic Disorders Partnership Network and plans for the 2018 Jeans for Genes Day campaign.
2.40pm	Gideon's Charter	**Susan Passmore** CEO Prader-Willi Syndrome Association UK	Susan Passmore will share Gideon's Charter and her team's plans for a campaign to secure the right support, in the right place, at the right time, within all educational settings for children and young people affected by Prader-Willi syndrome.
2.55pm	We have changed the law... and now it's personal!	**Carrick Brown** Senior Manager Care Services Newlife	Carrick Brown will talk about how Newlife is going to use the law established to protect those most vulnerable in our society to ensure access to equipment and change the landscape of statutory provision.
3.10pm	Providing a children's palliative care service in the community	**Chris Roys** Chief Executive Jessie May	Chris Roys will talk about the work of Jessie May nurses supporting babies, children and young people with life-limiting conditions, and their families, in their own homes and community.
3.25pm	Fighting injustice for disabled people	**Eric Appleby** Director **David Laurence** Partnerships Manager Disability Law Service	David Laurence will explain how Disability Law Service, in partnership with Genetic Disorders UK, is planning to provide free legal advice to individuals affected by a genetic disorder and their carers.
3.40pm	One single-minded goal: finding a cure for Duchenne muscular dystrophy	**Alex Smith** Founder Harrison's Fund	Alex Smith will reflect on the journey he has been on to raise the profile and funding for research into Duchenne muscular dystrophy since he placed a single, multi award-winning advertisement in the *Evening Standard* in May 2013.
3.55pm	Wrap-up	**Caroline Harding** Genetic Disorders UK	
4.00pm to 5.00pm		**AFTERNOON TEA**	