FIRST UK SMA RESEARCH CONSORTIUM LAUNCHED IN SMA AWARENESS WEEK

28th SEPTEMBER – 4th OCTOBER 2015

The UK SMA Research Consortium launches in Awareness Week and has come about as a result of significant recent progress, leading to clinical trials for potential new treatments but also a realisation that there is so much more we still need to know. Such as when and how to treat patients and how we can help develop successful treatments for people affected by all SMA types throughout their lives.

The Consortium will consist of world-class research/clinical experts in SMA and other neuromuscular conditions (such as Muscular Dystrophy and Motor Neuron Disease) based in Oxford, Edinburgh, London and Sheffield Universities. It will be led jointly by Professor Kevin Talbot (University of Oxford) and Professor Tom Gillingwater (University of Edinburgh). The intention is to form an initial nucleus that will, in time, draw together other UK scientists.

The main aims are to:

- Establish an internationally leading network of UK research collaborators who will develop and execute a coordinated SMA research program to maximise the impact of existing synergies.
- Develop existing drug targets and identify new neuroprotective therapies to maintain function throughout the life of SMA patients.
- Identify improved delivery methods for SMN and non-SMN based therapies.

The UK SMA Trust is funding a new £1.3m initiative for SMA research in the UK.
SMA Europe Activities

NEW GRANTS AWARDED
Through its 7th Call for Research Proposals, SMA Europe received 15 applications for research funding. The top 4 were chosen for funding, totalling €451,500, each developing a different field of SMA research.

The awardees were (in alphabetical order) Dr. Reuben Artero of Valencia University in Spain, Dr. Martine Barkats of the Institut de Myologie in France, Professor Francesco Muntoni of UCL in the UK, and Dr. Wilfried Rossol of Emory University in the US. For information on these projects, please visit our website.

STRATEGIC MEETING IN OXFORD
SMA Europe held its second summer meeting in Oxford on 10th and 11th July, jointly hosted by The SMA Trust and SMA Support UK.

Although primarily a strategic meeting, speakers were invited to present their work and potential collaborative ideas to the Board. In particular Professor Talbot and Dr. Mélissa Bowerman of Oxford University, Treat-NMD representatives Professor Eugenio Mercuri and Agata Robertson as well as Dr. Mariacristina Scoto and Danielle Ramsay of SMA REACH UK. For more information, please visit our website.

8TH CALL FOR RESEARCH PROPOSALS LAUNCHED
SMA Europe launched its 8th Call for Research Proposals this summer. Applications aimed at finding a therapy for SMA or at elucidating the basic pathophysiological processes of the disease, are sought from eligible scientists.

EUROPEAN SURVEY FOR PEOPLE AFFECTED BY SMA AND THEIR CARERS
SMA Europe initiated the first phase of a pan-European study to establish the patient perspective on meaningful therapeutic outcomes. Led by the French organisation, the Association Française contre les Myopathies (AFM), the project saw the distribution of a short survey across our member countries. At the time of writing, 586 surveys had been collected but a statement on outcome will be added to our website in due course.

Trends in Motor Neuron Pathobiology’ was held. In recent years, significant progress has been made in identifying potential molecular mechanisms underlying motor neuron diseases. This session explored whether commonality exists between them in order to identify novel drug approaches. Expert researchers who work in related motor neuron disorders, such as our

The Trust also represented SMA Europe at a pre-competitive industry meeting held for patient organisations, industry representatives and other stakeholders.

One of the highlights of the pre-competitive industry meeting for both The SMA Trust and SMA Europe, was a presentation by Elena Mazzone, Research Physiotherapist at the Catholic University’s Paediatric Neurology Unit in Rome, showcasing the work on motor scale refinement, which the two organisations funded. It was also a good opportunity to learn more about the FDA engagement plan on patient-focused drug development. This might prove a useful model from which to take inspiration when representing the patient view to our own regulators.

The conference agenda itself covered two and a half days of presentations, in addition to evening events which fostered researcher-family interaction. It was gratifying to see the significant number of projects being presented in the various sessions which had been funded by either The SMA Trust or SMA Europe.

In addition to the main research themes presented, a special session on ‘Emerging... and finally, potential similarities with Inherited Peripheral Nerve Disease with the function and transport of mitochondria (the cells’ ‘powerhouses’).
Update on UK projects

THE SMA TRUST CURRENTLY FUNDS OVER £1/2 MILLION IN LIVE PROJECTS HERE IN THE UK:

1 Professor Francesco Muntoni, UCL Institute of Child Health (in partnership with Great Ormond Street Hospital): SMA REACH UK
2 Dr. Heidi Fuller, University of Keele: The Molecular Consequences of Reduced SMN in iPSC-derived Human Motor Neurons
3 Professor Matthew Wood, University of Oxford*: Central Nervous System Delivery Peptides Conjugated to Oligonucleotides for Splice Switching Therapy of SMA
4 Professor Tom Gillingwater, University of Edinburgh*: Identifying and Protecting Vulnerable Motor Neurons in Spinal Muscular Atrophy

* In collaboration with Muscular Dystrophy UK

SMA Therapy Pipeline: August 2015

A potential new therapy for SMA has entered clinical development. Tirasemtiv, developed by Cytokinetics in the US, is a muscle activator which has shown increases in skeletal muscle force in response to neuronal input as well as delays in the onset and reduction in the degree of muscle fatigue. It is the seventh SMA drug program to advance to clinical trials.

By Treatment Target

- SMN2 Back-up Gene Enhancement
  - Identification
  - Optimisation
  - Safety & Manufacturing
  - Phase I Safety
  - Phase I Efficacy
  - Phase II Proof

- SMN1 Main Gene Replacement
  - Identification
  - Optimisation
  - Safety & Manufacturing
  - Phase I Safety
  - Phase I Efficacy
  - Phase II Proof

- Motor Neuron Protection
  - Identification
  - Optimisation
  - Safety & Manufacturing
  - Phase I Safety
  - Phase I Efficacy
  - Phase II Proof

- Muscle Enhancement
  - Identification
  - Optimisation
  - Safety & Manufacturing
  - Phase I Safety
  - Phase I Efficacy
  - Phase II Proof

In July, we launched our first e-research newsletter, Zoom in 2 SMA Research, where you can keep up-to-date with research developments as they happen.

FLASH NEWS UPDATES

In the pipeline...Dr. Groen’s Column

One of our talented young SMA scientists will soon be writing a column for forthcoming newsletters on the new trends in SMA research. In the meantime, find out more about Dr. Groen, by reading his short bio in the research section of our website www.smatrust.org
MAD CHALLENGE

TEAM NEWS

YET ANOTHER BUSY FEW MONTHS FOR OUR BRILLIANT FUNDRAISERS TAKING ON ALL SORTS OF CHALLENGES: FROM WALKS TO RUNS, FROM SCARBOROUGH TO STOCKHOLM – ALL #FUNDRAISING4SMA!

2015 is a ‘marathon’ year for The SMA Trust, with 9 brilliant runners taking on this particular kind of MAD challenge. Huge congratulations and thank you to Phil Lord (Team Arthur) – Midnight Mountain Marathon; Thomas Hasmond – Brighton Marathon; Mel Nimmons (Team Nelle) – Virgin London Marathon; Lily Nimmons (Team Nelle) – Essex marathon; Andy Shaw – Stockholm Marathon, Holly Forster, Rebecca Clayton, Laura Evans and Sarah Fitzsimmons – Rock n Roll Liverpool marathon. Between them they have raised over £8k!

Welcome to our new teams: Team Alexa: based in London, Alexa has Type 2 SMA. Friends and family have some brilliant MAD challenges planned, including their off-road cycling challenge on the South Downs Way and The Antarctic Ice Marathon in the South Pole. Team Emelia: Emelia lives in Oxfordshire and has Type 2 SMA. Her family and friends have many events planned including a BBQ and a race night.

FEBRUARY

Carrying on from our last newsletter (and continuing the great start to 2015), Team Carli held a fashion show jointly with M & Co raising a brilliant £535. The Lane family gave up some of their favourite things for Lent: Mandy gave up her personal Facebook page; Alan gave up coffee and Matthew gave up sweets; sadly Otis the dog also had to give up sausages!

MARCH

Playstock United Cotts Under 8’s team took to the pool in a sponsored swim to support Team Arthur, raising £640, while Grace Clarke from Team Oscar was sponsored to train for a professional fight which took place at a live fight night in Sheffield and which she WOW! Go Grace! Team Travis took in the beautiful views of Pen Y Fan to mark what would have been Travis’ 2nd birthday. Emily Cole (Team Nelle) took on the Colchester 10K in memory of a very beautiful little girl. Neil Friedman raised over £100 by shaving his beard and head.

APRIL

Team Lauren organised a charity fitness day: many people took part in Zumba favourite – Irish dancing. They raised £2530.

Our 2nd Metal Detecting Day was again a huge success raising £3326. Team Arthur had some keen runners this month with Ben Rennick, Henry Thomas (Plymouth Half Marathon) and Siobhan Nail (Windsor Half Marathon) all taking on amazing running challenges and raising lots of money. The lads from Canon Frome Cricket Club not only cycled a total of 16,000 miles over 3 days but also hosted a brilliant ball, with 50% of the proceeds coming to the Trust. Thank you also to Ellie Lawton, Vicky Bryant and Rebecca Parks who all took on their own challenges this month.

MAY

May saw The SMA Trust host the 2nd year of our cycling event, now newly named Ride Scorpion. 45 cyclists took on various routes and met at the finishing line at The SMA Trust’s original HQ in the glorious Compton Scorpion Estate in the Cotswolds. Team Isaac held a charity night in Ryhope which included a tombola, raffle and photo booth. Pupils from St Paul’s C of E Primary School held a non-uniform day and the Derwenthurst Club also held a race night. Andy Smale and friends (Team Arthur) took on the gruelling Plymouth Grand Fondo and Glynn Sanderson took part in the Gran Piccolo, a 60km Cycling Sportive.

Elisa Bostock from Team Henry ran in the Woodbridge 10k. Sara Gomes held a beauty event at The London College of Beauty Therapy. Eli Lawton ran the Leeds Half Marathon, raising over £300. 26 walkers joined Team Rowan for their annual Onesie Walk to celebrate what would have been Rowan’s 4th birthday and Team Layla raised a brilliant amount from their sunshine-filled golf day. Mod Corsham held a Race Night, raising a brilliant £700.

JUNE

We welcomed Lee Cameron fundraising for his sister Donna in the Cardiff Bay Triathlon and Raj Dosanjh ran the Toronto 15k for her niece who has SMA. Team Rowan held their second onesie event of the year with 46 children walking 2.5k at a local park. Emma Bowers walked 85 miles in memory of her nephew and Team Layla took in the beautiful scenery in their Great Glen Canoe Trial.

www.smatrust.org
JULY

July moved from 2 legs to 2 wheels, with July being all about the bikes for The SMA Trust! Devoted supporters of Team Rowan cycled 205 miles over 3 days unsupported and off-road, from Whitehaven to Scarborough, raising over £5k. Phil Roberts, also Team Rowan, did the Invictas Ashes 2015 Cycle Challenge, an epic 300 mile ride from Old Trafford to The Oval. The challenge took 4 days and was a great endurance test!

Clive John (Team Hollie) organised his Captain's Charity Day at Bewdley Pines Golf Club. Hollie herself was the special guest and the day exceeded all expectations, raising £4,000.

Matt Venning and friends from Team Alexa cycled 100 miles off-road on the South Downs Way. Read their wonderful story on our website.

Freestyle 360 Gym opened its doors for a summer fete with lots of activities and the most delicious locally sourced ice cream. A big thank you goes to Tim & Amy Spittle for organising and to Charlie for manning our SMA Trust stall on one of the hottest days of the summer!

Team Rowan, Team Lily-Bea, Jack Lane and Hugh Chambers all wore their SMA Trust running vests while taking in the sites of London as they ran the Vitality British 10k.

AUGUST

August saw Sian and Ben of Millers Memory cycle from Eastbourne to Ipswich ITFC with the aim of raising £3652 which equates to one pound for every day Sian and her family have been without Phil who had SMA Type 2. Matt Harman from Team Oscar bravely faced a Tough Mudder.

Helen Molesdale from Team Henry celebrated her 30th birthday by asking for donations instead of gifts and Megan Davies and her mum Lea walked up Ben Nevis. Debbie Donoghue, Broadway Golf Club, organised her Ladies Captain Charity Alliance, raising £440 as part of her year's fundraising for the Trust.

OTHER FUNDRAISING NEWS

Team SMAsh! has organised too many events to list in preparation for their Nepal Trek later this year...from 'Minions of Fun' to eating a 6-course meal of creepy crawlies! Gary Murning, an author who has Type 2 SMA kindly donated royalties from his first book – currently £225.

Team Arthur SMA Netball Team won the Plymouth and District League – congratulations to them! 8 months in, Richard Armour is still growing his hair and beard in support of Team Hollie!

Wow – what will the next few months bring? Thank you for all your amazing fundraising ideas and efforts!

COMING UP...

UK SMA AWARENESS WEEK
28th September – 4th October 2015

This is the first time we’ve concentrated all our activities into one week, which ties in with events happening in other European countries. August was SMA Awareness Month in the US so thanks to all of you who have already organised events to tie in with this.

The main focus for the Trust in UK Awareness Week this month is to launch the brand new UK Research Consortium, but we’re also teaming up with SMA Support UK to run a social media campaign based on the Rugby World Cup, the theme being...TACKLE SMA. Check our website and social media platforms for more information.

Or you could join in with our Coin Amnesty. Just choose a day during Awareness Week and get family, friends and colleagues to donate all their small change to make a big change to SMA research. See the website for details and a downloadable promotional poster.

We’ll also be drawing our Spring/Summer Raffle on 30th September. Thanks to everyone who has bought/sold so many tickets and here’s hoping your lucky number comes up. Winners will be posted on the website and we’ll also be notifying them personally.

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MUSCLE POWER DINNER, THE GLOBE THEATRE
Wednesday 14th October 2015

Tickets are now sold out for this glittering evening at The Underglobe at The Globe Theatre in London. Apart from raising awareness of SMA the dinner will also help us fund the new UK SMA Research Consortium. Our thanks go to everyone who has bought tables, donated auction prizes or sponsored this event, which promises to be a truly memorable evening!

Get involved...

CALENDAR OF EVENTS

Have a look at our Events Calendar to see how you can join in with events either organised by us or by other organisations that we’re working with. We’re currently in the process of finalising plans from Spring 2016 onwards, so keep checking for the most up to date information:

www.smatrust.org/get-involved/mad-challenge-events

COMMUNITY ROADSHOWS

We’re keen to continue our programme of Community Roadshows and would like to come and meet more of our fundraising teams. This is an opportunity to get to know more of our brilliant supporters, tell them about the difference their support is making and thank them in person. Do get in touch if you would like to arrange an event in your area.

1000 MILE CHALLENGE

This is a new way you can help us raise vital funds for our Muscle Power campaign. All you have to do is travel 1000 miles – there’s no time limit and no limit on how many people you get to help you; you certainly don’t have to do it on your own! So you can get on your bike, row your boat, walk the dog, ride your horse...just about anything you like to get to that magic target!

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SRIN MADIPALLI

Q. When were you diagnosed with SMA and how does it affect you now?
A. I was diagnosed when I was two. Now I use a powered wheelchair and require 24/7 care. I have two PAs who help me live independently here in London. I don't have any movement in my legs but can use my hands a little bit. However, I like to think SMA doesn't stop me living the life I aspire to have.

I’m an entrepreneur running a tech start-up called Accomable that aims to be the ‘Expedia for accessible travel’. I’m also a qualified lawyer with degrees from Kings College London and Oxford University. I’ve travelled the world and always lived life to the full, regardless of difficulties and challenges.

Q. What is a typical day for you?
A. My days are pretty crazy at the moment as our start-up is beginning to grow. I’m usually up by 8am and keep going till about 2am; I normally work 7 days a week (yes, I’m a workaholic and need to keep busy as I struggle to do nothing!). I try to spend the early part of the day on calls and meetings. As the main developer/coder on our app, afternoons and evenings are usually spent dealing with technical issues or working on new features and improvements. Even though I work hard, I still make sure I spend as much time as I can with my awesome circle of friends and family. I make a point of hanging out 2 or 3 evenings a week with them.

Q. How did you get involved with The SMA Trust and why did you decide to become a Trustee?
A. I first came across the Trust 5 years ago when I was invited to talk at an event. Helping our community of individuals and families living with SMA is very important to me; and for a long time I’d been keen to get involved with a charity that had similar goals. I also wanted to make sure that people with SMA have a voice in key organisations that affect their daily lives. So when the opportunity to become a Trustee became available, I jumped at the chance!

Q. What has been your biggest achievement/are you most proud of?
A. A mix of things! Academically, it would either be graduating first in my class or getting into Oxford as a post-grad. Professionally, I’d have to say either qualifying as a solicitor or learning to code; and on a personal level, travelling around the world!

Q. What do you like doing in your spare time?
A. I love spending time with friends and just going out to eat, drink, go to the cinema etc. I’m quite a nerdy character so I enjoy reading, visiting exhibitions and using my time to learn new things. I also travel a lot and have a big sense of adventure. I’ve scuba-dived, camped out in the wilderness and done a whole bunch of fun things around the world!

Hollywood comes to Dudley for their SMA Ball!

This was the 3rd year of this event, organised by Toby and Paula Flatman, whose daughter Hollie has Type 3 SMA.

This year saw a Hollywood theme, with disconcertingly life-like celebrity masks that added to the star-studded atmosphere. However, this ball isn’t just about fundraising and the real stars were the families affected by SMA who came from all over the UK, as well as Ireland and Austria, to enjoy meeting up, sharing their experiences and having fun! The event has grown over the 3 years, from 120 guests raising £4,200 to 350 guests this year, raising over £11,000! Plans are already in place for next year’s event. The theme is Mad Hatter’s Tea Party and it will be held once again at the Copthorne Hotel – there’s already been interest from as far away as Iceland.

A BIG thank-you to the Flatmans, as well as their amazing team of friends and family, who all do their bit to help to make this event such a success in every sense.

www.smatrust.org

SMA is the leading genetic killer of infants and toddlers
The SMA Trust is stepping up nationally and internationally

Joanna Mitchell, Chief Executive

I hope you’ve enjoyed reading highlights from another exciting few months for the Trust.

The SMA REACH Project we funded through Great Ormond Street Hospital has resulted in the development of a new international alliance between centres in the UK, Italy and the US. This collaboration has resulted in further improvements to the motor function scales used in clinical trials, together with a patient focus group programme to establish how small improvements on the scale can make a significant difference to people with differing severity of SMA.

As the Trust and within SMA Europe, we continue to build closer relationships with the pharmaceutical industry and regulatory authorities, especially EMA (European Medicines Agency). It is encouraging to see that there are now 7 companies with projects in the clinical development phase. This year we’re linking with other European countries and celebrating the UK’s first SMA Awareness Week (28th September - 4th October). We will be using the week to formally launch the new Consortium but have also been working closely with SMA Support UK on other activities and have planned, amongst other things, a social media campaign with a rugby theme to tie in with the Rugby World Cup... #Tackle SMA.

We’re also encouraging people to help raise awareness (and funds) during Awareness Week by organising a Coin Amnesty, the idea being for companies and other groups to donate all that annoying loose change to The SMA Trust.

Our MAD Challenge teams continue to inspire us all with their blend of passion, ideas and sheer determination. Last financial year they raised a phenomenal £194,000 for the Trust and are already on course for a substantial increase this year. We’re so grateful to them and all of you for everything you do to help us continue our work. The calls on our funds and time are increasing rapidly at the moment and we’ve had to put ambitious fundraising growth targets in place over the next 3 years, to make sure we can honour existing commitments, but also be open to new initiatives.

There’s so much to be done. Let’s do it together!

The SMA Trust Team

There have been some changes to the team in recent months.

We’ve said goodbye and a big thank you to Mandy Lane and Ginny Cullen (although many of you will continue to see Mandy as she’s promised to still help out at events when she can); and we welcome Mary Boullin and Alison Braithwaite who join us as Community & Events Fundraising Manager and Part-time Marketing & Communications Co-ordinator respectively. We will be recruiting a new Trusts, Corporate & Major Gifts Fundraiser in the Autumn.

Sarah Poole, who has been done a wonderful job of helping out with Social Media for a while, has officially joined the team working 2 days a week on social media and fundraising support.

Mary Boullin

Community & Events Fundraising Manager

Mary joins us from Helen & Douglas House Hospice for Children & Young Adults, where she was Head of Community and Events Fundraising. Before getting into fundraising, Mary’s previous experience included working as a learning mentor for young people in care.

“I am really looking forward to working for The SMA Trust. Apart from my professional fundraising experience, I also bring baking skills, plus negotiating skills learnt from managing my slightly hyper dog Frankie, who will be joining me in the office and offering her support to the team.”

Alison Braithwaite

Marketing & Communications Co-ordinator

Alison has significant marketing and comms experience in both the corporate and charity sectors and will help us achieve our ambitions to increase awareness of SMA and The SMA Trust across all communication platforms.

“I’m delighted to be joining The SMA Trust team and looking forward to putting my marketing skills to good use for the benefit of SMA Research.”

Sarah Poole

Fundraising Support & Social Media

Sarah (along with her husband Matt) first got involved when they started up Team Rowan, in memory of their little girl who died of Type 1 SMA in 2012. Not only does the team continue to raise amazing amounts of money for the Trust, but we’re now lucky to have Sarah on board, inspiring and supporting other fundraising teams in their efforts.

“When we lost Rowan I wanted to do everything possible to help raise awareness and fundraise for research into SMA. I find supporting other families affected by SMA who want to fundraise for The SMA Trust so rewarding.”

Please turnover for all our contact details

www.smatrust.org
I WOULD LIKE TO MAKE A
REGULAR GIFT OR DONATION

1 YOUR DETAILS
Title: First Name:
Last Name:
Address:
Postcode:
Telephone:
Mobile:
Email:

2 MAKE A REGULAR GIFT
I would like to make a regular gift of
£
Starting on
and the same sum on the same day monthly
Please set up a standing order with your bank to:
Lloyds TSB, Stratford upon Avon
(Sort Code 30-98-26)
The SMA Trust
(Account No. 02628431)
Signature:
Date:

3 MAKE A DONATION
I would like to make a one off donation
to The SMA Trust of
£
☐ I have enclosed a cheque made payable
to The SMA Trust

4 GIFT AID
giftaid it
Gift Aid means that for every pound you give, we receive an extra 25p from the Inland Revenue, helping your gift go even further.
☐ Yes I want all donations I have made since 6th April 2008, and all donations I make in the future, to be eligible for Gift Aid. Please tick the box above.

Please return this form to:
The SMA Trust 1c Atherstone Barns, Atherstone on Stour, Stratford-upon-Avon, Warwickshire CV37 8NE

THANK YOU FOR YOUR SUPPORT