

The Conference will take place over two days on Friday November 4th and Saturday November 5th at the Holiday Inn, Coram St, Bloomsbury, London WC1N 1TH from 9.00am until 6.00pm.

There will be key note speakers, major presentations, breakout sessions and workshops. The final agenda will be published on our website.

The cost for a full two day ticket including entry to all meetings, Lunch both days and Friday Night Dinner and Cabaret is £60. Free Creche is available for 12 years and under.

\*\*\*\*One person FREE Special Offer for Action Duchenne members only - £60 will cover the cost of a member plus a guest. Remember to book with your membership number\*\*\*\*

To book, and for more information about upcoming sessions, please visit our website -

<http://www.actionduchenne.org/conference2011>



By inviting research groups, clinical experts or biotech companies to our annual Conference to present their data and express their opinions is not an endorsement of any particular therapy, procedure, drug, or device. The Conference is open to families to attend so that they may learn more about the progress being made to treat and support those living with Duchenne Muscular Dystrophy. Action Duchenne strongly recommends that families and patients only undertake a course of drugs or therapy after consulting specialist medical advice. Families in the UK can locate their nearest specialist muscle centre by going to [www.dmdcentres.org](http://www.dmdcentres.org) and ask their GP to be referred for consultation.



#### CONTACT US:

Action Duchenne  
Epicentre  
41 West Street  
London  
E11 4LJ  
Tel: 0208 556 9955  
Email: [kelly@actionduchenne.org](mailto:kelly@actionduchenne.org)  
Web: [www.actionduchenne.org](http://www.actionduchenne.org)

Registered Charity No: 1101971



# IT'S TIME TO STOP WASTING!

## 9th International Annual Duchenne Conference

Friday 4th November 9am – 6pm Gala Dinner at 7.30pm  
Saturday 5th November 9am – 6pm

Register online at  
[www.actionduchenne.org/conference2011](http://www.actionduchenne.org/conference2011)



Holiday Inn  
Coram St Bloomsbury London

## FRIDAY 4 NOVEMBER

Time	8.00 - 8.50	9.00 - 9.30	9.30 - 10.00	10.00 - 10.30	10.30 - 11.00	11.00 - 11.30	11.30 - 12.00	12.00 - 12.30	12.30 - 1.00	1.00 - 2.00	2.00 - 2.30	2.30 - 3.00	3.00 - 3.30	3.30 - 4.00	4.00 - 4.30	4.30 - 5.00	5.00 - 5.30	5.30 - 6.00
Turner (150)	Registration Tea & Coffee	There is an Elephant in the room - Progress of Duchenne Research Dr Karl Bettelheim Action Duchenne	What do NHS Reforms mean for patient care? - Changing the map for Duchenne Arlene Wilkie, Chief Executive, Neurological Alliance, Alastair Kent, Chair of Rare Disease UK, Eilidh Macpherson Action Duchenne	Coffee	Biomarkers for clinical trials Dr Sebahattin Cirak UCL	Introduction to Exon Skipping Prof Steve Wilton Western Australia	Improving the design of antisense drugs using peptides - new clinical trials Dr Mike Gait University of Cambridge, Professor Matthew Wood University of Oxford		Lunch	Using AAV vectors and microdystrophin Prof George Dickson Royal Holloway	MDEX Clinical Trial Programme and Study 28 Professor Francesco Muntoni Great Ormond Street London	AVI 4658 Clinical Trials Dr Ed Kaye AVI	Coffee	Action of ACE inhibitor perindopril on cardio skeletal muscle in DMD - basic and clinical results Dr Denis Duboc France	Revatio for heart disease in DBMD - Pilot trials now clinical trial Dr Dan Judge USA	IGF-1 therapy and muscle function in Duchenne Dr Meilan Rutter Cincinnati USA		
Booker (150)	Registration Tea & Coffee			Caring for the heart Dr John Bourke Newcastle	March of the young men Gordon McClurg CMS Newcastle	Respiratory care Dr Michelle Eagle Newcastle	Duchenne Alliance Christine McSherry Jett Foundation USA and Carlo Rago USA	Bone protection for children with DMD treated with corticosteroids Dr Ros Quinliven Great Ormond Street London	Endocrinology and Duchenne Dr Meilan Rutter Cincinnati USA	Peptide based inhibition of NF-kB Professor Paul Jansen Ohio USA	The holy grail of multiple exon skipping Professor Terry Partridge	Catena for Duchenne Muscular Dystrophy Dr Nicholas Coppard Santhera Switzerland	Early treatment with lisinopril & spironolactone for the heart Professor Paul Jansen Ohio USA					
Jasmine (50)	Registration Tea & Coffee				DMD Registry Steering Committee Meeting Dr Steve Abbs and Angela Stringer		Independent living workshop Care Management Services			The Vector Machine - the need for a clinical trial Dr Donald Rhodes USA								
Grammy (50)					Genius Comedy workshops for young people for 12+yrs													
Nobel (50)					Newly diagnosed families Tony Levene, Divyesh Popat (trustees) and Rachel Salmon Care Officer for Wales	DMD centres - working with your specialist team Eilidh Macpherson Action Duchenne			Join the DMD Registry - UK Patient Registry for clinical trials Angela Stringer DMD Registry Curator	Problems at school - advice and discussion Janet Hoskin Action Duchenne and James Poysky USA			Letting go - parents of young people 13+ Janet Hoskin Action Duchenne and Celine Barry Action Duchenne					
Diploma and Catey					Creche for under 12s													

7.30pm - Gala dinner in Turner and Booker hosted by Francesca Martinez

## SATURDAY 5 NOVEMBER

Time	8.00 - 8.50	9.00 - 9.30	9.30 - 10.00	10.00 - 10.30	10.30 - 11.00	11.00 - 11.30	11.30 - 12.00	12.00 - 12.30	12.30 - 1.00	1.00 - 2.00	2.00 - 2.30	2.30 - 3.00	3.00 - 3.30	3.30 - 4.00	4.00 - 4.30	4.30 - 5.00	5.00 - 5.30	5.30 - 6.00
Turner (150)	Registration Tea & Coffee	Light at the end of the tunnel Dame Professor Kay Davies University of Oxford	Debate: "Clinical trial protocols are no longer fit for purpose - N=1 provides a radical alternative" Dr Annemeike Rus, Nick Catlin CEO Action Duchenne, Chair John Henderson Action Duchenne Trustee	Coffee	Targeting therapeutic molecules to mitochondria Dr Mike Murphy University of Cambridge	Biglycan as a therapy for Duchenne Dr Justin Fallon USA	Expectations of clinical trials Susie Dorricott Prosensa Netherlands	Update on SMT1100 a utrophin upregulator for Duchenne Dr Jon Tinsley Summit PLC	Lunch	A review of Prosensa's exon skipping programme Dr Giles Campion Prosensa Netherlands	Personalised medicines for Duchenne Padraig Wright Vice President Clinical Neurosciences at GlaxoSmithKline	Stem cells for DMD Dr Jenny Morgan UCL London, Professor Terry Partridge Washington USA	Coffee	A new generation of drugs to treat Duchenne? Chair Francesco Muntoni plus panel of conference speakers. Submit any questions from presentations at the conference				
Booker (150)	Registration Tea & Coffee			Physiotherapy Marion Main Great Ormond Street Hospital	Discovering drugs for Duchenne Dr Ellen Welch PTC Therapeutics	AAV-based mRNA therapies for Duchenne muscular dystrophy Dr Luis Garcia France	Enabling DMD clinical trials with large-scale production of AAV vectors Dr Robert Kotin NIH USA	Learning and Behaviour Problems in Duchenne - Include Duchenne Project Dr James Poysky USA and Janet Hoskin Decipha	Takin' Charge - living a full life Dr Jes Rahbek Denmark									
Jasmine (50)	Registration Tea & Coffee				Progress of Duchenne research Dr Karl Bettelheim		Takin' charge for young people living with Duchenne Janet Hoskin Action Duchenne and Celine Barry Action Duchenne			Join the DMD Registry - UK Patient Registry for clinical trials Angela Stringer DMD Registry Curator	MDEX Study 28 Briefing for Families taking part Professor Francesco Muntoni MDEX			Duchenne with a future: The power to Live Documentary film with Jos Hendriksen Netherlands				
Nobel (50)					Campaigning for Duchenne with Eilidh Macpherson Action Duchenne and National Advocacy Council members		Physiotherapy Workshop Marion Main Great Ormond Street			Duchenne for dads Tony Levene, Divyesh Popat, Paul Ackroyd (trustees) and Rachel Salmon Care Officer for Wales								
Grammy (50)					Genius Comedy workshops for young people for 12+yrs													
Diploma and Catey					Creche for under 12s													

Information correct at time of going to press.