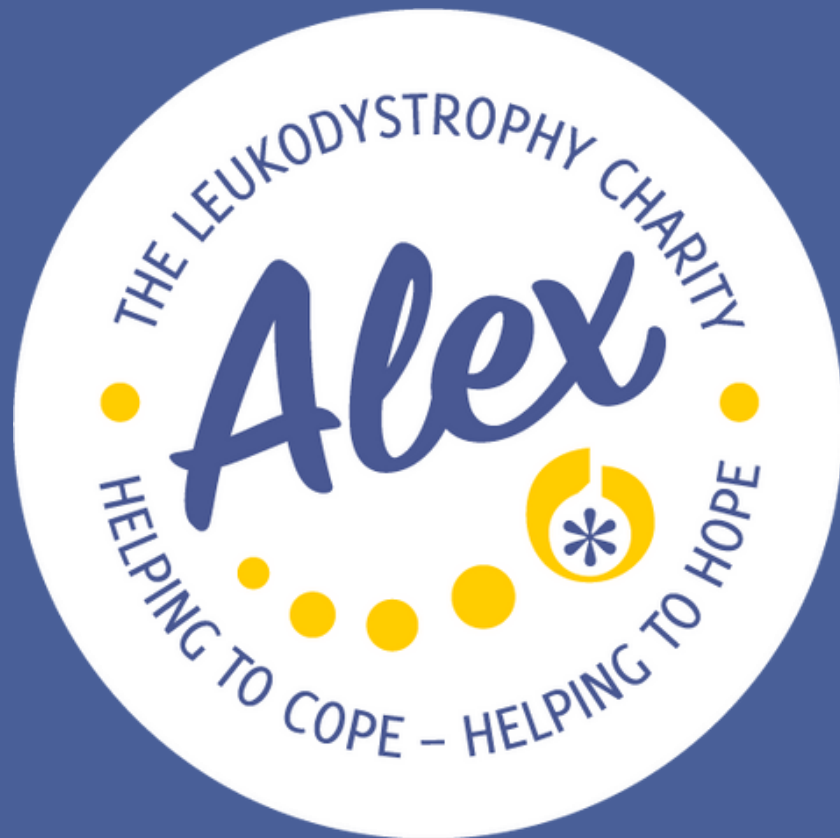


# Alex, The Leukodystrophy Charity



## Community Weekend Agenda

28th April – 1st May 2023  
Hilton Birmingham Metropole

# Weekend Agenda Overview



## Friday

Time	Activity	Room
15:00-18:00	Check In to Hotel and Visit Welcome Table	Hotel Foyer
18:30-22:00	Dinner and Evening Entertainment	Monarch Suite

## Saturday

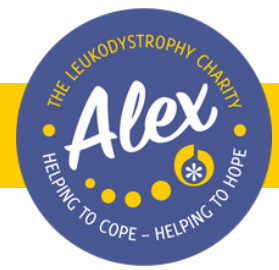
Time	Activity	Room
09:30-15:15	Presentations	Sovereign
09:30-17:45	Zebra Fish Project	Viscount
11:35-12:50	Presentations	Norfolk
13:10-14:00	Lunch	Viscount
15:45-17:45	Workshops	Sovereign
15:45-17:45	Research Roundtable (professionals only)	Norfolk
18:45-22:00	Dinner and Evening Entertainment	Monarch Suite

## Sunday

Time	Activity	Room
09:30-15:15	Presentations	Sovereign
11:40-12:55	Presentations	Norfolk
12:55-13:45	Lunch	Viscount
15:45-17:45	Workshops	Sovereign
15:45-17:45	ALD Newborn Screening Roundtable (professionals only)	Norfolk
18:45-22:00	Dinner and Evening Entertainment	Monarch Suite



# Weekend Agenda: Saturday



## Presentations

Time	Topic	Presenter	Room
09:30-09:35	Alex TLC Introductions	Sara Hunt	Sovereign
09:35-09:55	Alex TLC Update	Sara Hunt	Sovereign
09:55-10:15	NHS England Inherited White Matter Disorder (IWMD) Service and Patient Registry	Dr Rahul Singh	Sovereign
10:15-10:35	Working with Alex TLC - NHSE IWMD Service & Registry	Helen Morris (Metabolic Support) & Toni Mees	Sovereign
10:35-10:55	Leukolabs - a UK Research Collaboration	Dr Noémie Hamilton	Sovereign
10:55-11:10	Q&A		Sovereign
11:10-11:35	Break		Viscount
11:35-11:55	Gene Therapy/Bone Marrow Transplant	Dr Claire Horgan	Sovereign
11:35-11:55	Experience of being a young carrier	Remember the Girls (Taylor Kane)	Norfolk
11:55-12:15	Adrenomyeloneuropathy (AMN) - Symptoms and treatments for men	Dr Robin Lachman	Sovereign
11:55-12:15	Symptoms and Treatments in Women with Adrenoleukodystrophy (ALD)	Dr Florian Eichler	Norfolk
12:15-12:35	Transplant for Advanced Cerebral ALD: Risk-Benefit	Dr Troy Lund	Sovereign
12:15-12:35	Quality of life and lifestyle intervention for women with ALD	Dr Wolfgang Kohler	Norfolk
12:35-12:55	Support for severely symptomatic patients (adults)	Dr Charlie Wade & Prof. Jeremy Chattaway	Sovereign
12:35-12:50	Q&A		Norfolk
12:55-13:10	Q&A		Sovereign
13:10-14:00	Lunch		Viscount
14:00-14:20	The psychosocial impact on parents of having a child diagnosed with cerebral ALD	Dr Hilary Piercy	Sovereign
14:20-14:40	ALD: Monitoring (children)	Dr James Davison	Sovereign
14:40-15:00	Hematopoietic stem-cell transplantation (HSCT) in Adults with Adult Onset Cerebral ALD	Dr Wolfgang Kohler	Sovereign
15:00-15:15	Q&A		Sovereign
15:15-15:45	Break		Viscount



# Weekend Agenda: Saturday



## Workshops

Time	Topic	Presenter	Room
09:30-17:45	Zebra Fish Project	Dr Noémie Hamilton & Team	Viscount
15:45-16:45	Project Development with Alex TLC	Marlene Graham & Suzanne Gosney	Sovereign
16:45-17:45	Community Connections - a place to meet and chat	Kathleen O'Sullivan-Fortin & Karen Harrison	Sovereign

## Research Roundtable (professionals only)

Time	Topic	Presenter	Room
15:45-17:45	Canavan Update MLD Update ALSP Update AGS Update Krabbe Update Roundtable chaired by Dr Noémie Hamilton, Leukolabs	Dr Florian Eichler Dr Dipak Ram Dr Charlie Wade Dr Yanick Crow Michele Salvo	Norfolk

## Children's Activities

Time	Activity	Presenter	Room
09:00-10:00	Art Room	Alex TLC Team	Surrey
10:00-13:00	Clay Pottery	Creative Active Lives	Surrey
14:00-17:45	Accessible Circus Skills	Circus Allstars	Viscount



# Weekend Agenda: Sunday



## Presentations

Time	Topic	Presenter	Room
09:30-09:35	Alex TLC Introductions	Karen Harrison	Sovereign
09:35-09:55	Summary of Saturday's Research Roundtable	Dr James Poulter (Leukolabs)	Sovereign
09:55-10:15	Sex-specific newborn screening for X-linked Adrenoleukodystrophy (ALD)	Dr Stephan Kemp	Sovereign
10:15-10:35	Alex TLC Newborn Screening Study	Genetic Alliance - Jennifer Jones	Sovereign
10:35-10:55	Gene Therapy for Adrenomyeloneuropathy (AMN)	Dr Florian Eichler	Sovereign
10:55-11:10	Q&A		Sovereign
11:10-11:40	Break		Viscount
11:40-12:00	Genetics & Genetic Counselling	Dr James Poulter	Sovereign
11:40-12:00	How to cope with a long term condition	Dr Pujit Gandhi	Norfolk
12:00-12:20	Paediatric Symptom Management	Dr Alasdair Parker	Sovereign
12:00-12:20	Queen Square Adult Leukodystrophy Group	Dr David Lynch	Norfolk
12:20-12:40	ALD Monitoring - Personal Story	Kathleen O'Sullivan-Fortin	Sovereign
12:20-12:40	Bladder Care	Dr Sarah Wright	Norfolk
12:40-12:55	Q&A		Sovereign
12:40-12:55	Q&A		Norfolk
12:55-13:45	Lunch		Viscount
13:45-14:15	Minoryx Therapeutics S.L. Clinical Programme Update	Dr Arun Mistry, Minoryx Therapeutics	Sovereign
14:15-14:30	SwanBio Program Updates: investigating AAV gene therapy for AMN	Eileen Sawyer, SwanBio	Sovereign
14:30-15:00	VK0214 in Patients with AMN – Update	Dr Joel Neutel, Viking Therapeutics	Sovereign
15:00-15:15	Q&A		Sovereign
15:15-15:45	Break		Viscount



# Weekend Agenda: Sunday



## Workshops

Time	Topic	Presenter	Room
15:45-16:45	Get Involved in Alex TLC	Marlene Graham & Suzanne Gosney	Sovereign
16:45-17:45	Mindfulness	Dr Pujit Gandhi	Sovereign

## ALD Newborn Screening Roundtable (professionals only)

Time	Topic	Chair	Room
15:45-17:45	2024 National Screening Committee Review	Dr Robin Lachman	Norfolk

## Children's Activities

Time	Activity	Presenter	Room
09:00-10:00	Art Room	Alex TLC Team	Surrey
10:00-13:00	Craft Workshop	Creative Active Lives	Surrey
14:00-17:00	Bowling	Alex TLC Team	Surrey
17:00-17:45	Art Room	Alex TLC Team	Surrey

