



ERN-EYE annual WORKSHOP 2019
Progress and challenges in treating inherited retinal diseases
21-22 November 2019, Strasbourg, France

Meeting Location: CCI Alsace, 10 place Gutenberg, Strasbourg

Thursday – 21st November 2019

- 8:30-9:10** Registration of participants
- 9:10-9:20** Meeting opening: objectives of the workshop **Hélène Dollfus**, ERN-EYE Coordinator, Strasbourg, France & **Dominique Sturz**, ePAG representative, Kitzersdorf, Austria

SESSION 1	ADVANCES AND CHALLENGES in treating inherited retinal diseases
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- 9:20-9:30** **Introduction “What are the therapeutic challenges for posterior segment inherited dystrophies?”** **Bart Leroy**, University Hospital, Ghent, Belgium
- 9:30-10:00** **“Leber’s congenital amaurosis and phenotypic variability and biomarkers: Who and when to treat?”** **Birgit Lorenz**, Justus-Liebig-University, Giessen, Germany
- 10:00-10:45** **“Gene Therapy for Inherited Retinal Dystrophies: Current and future State of Affairs including the long term follow up in the post market era in the USA”** **Tomas Aleman**, Scheie Eye Institute, Philadelphia, USA
- 10:45-11:15** **“The Oxford experience with gene therapy; paving the way to bring gene therapy for X-Linked Retinal Dystrophies”** **Susan Downes**, John Radcliffe Hospital, Oxford, UK
- 11:15-11:45** **Coffee Break**
- 11:45-12:15** **“CRISPR/Cas-9 Technology for inherited retinal dystrophies”** **Eric Pierce**, Harvard University, Boston, USA
- 12:15-12:45** **“The eco-system of innovative therapies”** **Florence Allouche**, Faculté de Pharmacie, Université Paris Descartes & Sparing Vision, Paris, France
- 12:45-13:45** **Lunch**

SESSION 2	ERN WORKING SESSION ERN member state situation for clinical trials
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13:45-16:05 **“Situation of each member state for ERN-EYE”**

Belgium	France	Latvia	Portugal
Czech republic	Germany	Lithuania	United Kingdom
Denmark	Italy	Netherlands	Switzerland
Estonia	Ireland	Poland	

(delegate to be confirmed. 7min each, 2min discussion)

- 16:05-16:20** **“EVI.CRNet role in clinical trials in Europe”** **Cecilia Martinho**, EVICR.net, Coimbra, Portugal
- 16:20-16:50** **Coffee Break**



SESSION 3	ROUND TABLE (Part 1) Challenges and state of the art of current clinical studies in Europe (pre market)
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(15 min + 5 min discussion)

16:50-17:10	“QR-110 for CEP290-related LCA” <u>Bart Leroy</u> , University Hospital, Ghent, Belgium
17:10-17:30	“QR-421a for USH2A-related Retinal Dystrophies” <u>Isabelle Audo</u> , Institut de la Vision, Paris, France
17:30-17:50	“Lentivirus-based Treatments inherited retinal dystrophies” <u>Isabelle Audo</u> , Institut de la Vision, Paris, France
17:50-18:10	“X-linked RP and achromatopsia trials - MEH UK” <u>Michael Michaelides</u> , Moorfields Eye Hospital, London, UK
18:10-18:30	“Usher syndrome trials” <u>Francesca Simonelli</u> , Università degli Studi della Campania, Naples, Italy
18:30-18:50	“Achromatopsia” <u>Susanne Kohl</u> , University of Tübingen, Tübingen , Germany
20h	Dinner Maison KAMMERZELL

Friday – 22nd November 2019

SESSION 4	Patient’s expectations and realistic outcomes
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8:10-8:30	“What are the patients’ and parents’ perspectives” <u>Christina Fasser</u> , Retina International, Zurich, Switzerland
8:30-8:50	“Patient reported outcomes in visual research” <u>Orla Galvin</u> , Retina International, Dublin, Ireland
8:50-9:10	“Image based outcomes in retinal trial: how reliable?” <u>Philipp Herrmann</u> , University Hospital, Bonn, Germany
9:10-9:30	“Pediatric specificities in trials” <u>Elise Héon</u> , Hospital for Sick Children, Toronto, Canada
9:30-10:00	“The patient and Therapy Timing perspectives” <u>Hendrik Scholl</u> , University Hospital, Basel, Switzerland, TBC
10:00-10:30	Coffee Break

SESSION 5	ROUND TABLE (Part 2) Challenges of current clinical studies and post market in Europe: state of the art – where are we?
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10:30-10:45	“LUXTURNA the XV-XX experience; getting on the market” <u>Saddek Mohand Said</u> , CHNO des Quinze-Vingts, Paris, France
10:45-11:00	“Therapies for optic nerve disorders: experience in leber hereditary optic neuropathy” <u>Patrick Yu Wai Man</u> , Moorfields Eye Hospital, London, UK
11:00-11:15	“LHON gene therapy advances & challenges” <u>José-Alain SAHEL</u> , Institut de la Vision, Paris, France

ERN-EYE joint satellite meeting organized by FHU NeurogenyCS

11:20-11:30 *“Objectives of the satellite meeting FHU NeurogenyCS and ERN-EYE”* (HD-BL)

SESSION 1	Introduction novel therapies, new costs and challenges
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11:30-11:50 *“IRD COUNTS”* **Orla Galvin**, Retina International, Dublin, Ireland

11:50-12:10 *“The legal aspects on getting onto the market in the EU”* **Harald Heemstra**, Patient Access Director Rare Diseases Europe, NOVARTIS, Camberley, UK

12:10-12:30 *“The Rare Impact Initiative”* **Simone Boselli**, EURORDIS, Brussels, Belgium, **TBC**

12:30-12:50 *“Challenges raised by innovative therapies”* **Sandy Tubeuf**, Université Catholique, Louvain, Belgium

12:50-13:50 **Lunch**

SESSION 2	ROUNDTABLE Interactions with industry: the patient, the clinician and the market
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13:50-14:10 **Sue Lacey**, Director, Global Medical Affairs, Retina, NOVARTIS, Camberley, UK

14:10-14:30 **Aniz Girach**, Chief Medical Officer, ProQR, Leiden, The Netherlands

14:30-14:50 **Sascha Fauser**, Head of Ophthalmology, ROCHE, Bâle, Switzerland

14:50-15:10 **Magalie Taiel**, Chief Medical Officer, GENSIGHT BIOLOGICS, Paris, France

15:10-15:30 **Jean-Yves Deslandes**, Chief Medical Officer, HORAMA, Paris, France

15:30-16:00 **Coffee Break**

SESSION 3	ROUNDTABLE Retinal implants – past and future: where are we going to (or not)?
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16:00-16:20 *“The state of the Art”* **Serge Picaud**, Institut de la Vision, Paris, France

16:20-16:40 *“The retinal implant experience”* **Eberhart Zrenner**, University Eye Hospital, Tübingen, Germany

16:40-17:00 *“Retinal Implants with ARGus II”* **Maura Arsiero**, Director Business Development Western Europe, Second Sight, Lausanne, Switzerland

17:00 **End of the meeting**