



Seeking A Cure For  
Retinitis Pigmentosa, Macular Degeneration,  
Usher Syndrome and Allied Retinal Dystrophies

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## **Retina International General Assembly 2018 - Auckland, New Zealand**

Thursday, February 8th, 2018

Location: University of Auckland Business School

Owen G Glenn Building, 12 Grafton Road, Auckland, 1010

Room: Decima Glenn Room, Level 3

Start: 09.00 a.m. end: 05.00 p.m.

### **APPENDIX 06-1**

#### 6.1 Retina International Secretariat: Report of the CEO

#### **Motion: the MC moves to accept the CEO Report.**

#### **CEO Report:**

The Retina International secretariat implements an agreed work plan developed in collaboration with the management committee (MC), president and the CEO. The following is a brief report on work to date.

#### **Ongoing Projects Financed by Grants:**

In the stated period there are three ongoing projects financed by grants these are as follows:

#### **PROM - Patient Reported Outcome Measures**

Patient Reported Outcome Measures are critical in measuring the impact of treatments on patients as accurately as possible. In November 2015 RI received funding from Novartis to run a preliminary multi-stakeholder workshop in Dublin, Ireland. The meeting recommended a 'White Paper' be published on the importance of PROMs in developing mechanisms for

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access to and reimbursement of potential therapies for Retinal Degenerative Diseases. Novartis have funded further work on the project and so RI organised an open workshop entitled **-Functional Vision versus Visual Function - Working Towards Integrating the Patient Perspective-** in Washington DC on Nov 10, 2016. The meeting had participation from the FDA, EMA and NEI, Retinal specialists and Principle Investigators working in this field as well as patient organisations and industry representatives. A further meeting with representatives from this group was held on May 9<sup>th</sup> 2017 at the ARVO conference in Baltimore MD.

The organising committee made up of Prof. Eberhart Zrenner, Prof Elise Heon, Prof Jerry Chader, Ms. Christina Fasser and Ms. Avril Daly are now working with the representative group on development of the white paper and on securing a suitable publication for the project. Further meetings will take place in 2018 with a publication later in the year.

### **Genetic Testing On-line Toolkit**

SPARK Therapeutics funded the development of an online toolkit on genetic testing for IRDs. The toolkit is entitled **RED Alert** playing on the term Rare Eye Disease and taking ownership of this term for RI.

The Toolkit consists of text describing the fundamentals of genetics, the evolution of testing services, the types of tests available and where they can be accessed. There is also a section on the risk and the benefit the important issues to be considered and the importance of genetic counselling services. The toolkit has followed best practice and each text section is kept short and digestible but we have included in each an explanatory accompanying video clip in the hope that these will be of benefit to those who are vision impaired.

A survey of patients and member organisation has allowed us to understand the areas where there was a clear lack of information and tailor the toolkit to meet that particular need.

The text has been reviewed by three scientists and one clinician. A group of individuals with various IRDs and a group of young mothers with no knowledge of IRDs. This is to ensure that the information is useful to those who are aware of the basics and that it is not frightening or not understandable to those who are newly diagnosed.

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There toolkit was presented at EU Retina formally launched to our members on World Retina Day 2017. The toolkit has already been updated and new video clips and information is added. This is the first step in RIs objective of advocating better access to genetic testing services which is fundamental to our work and the project will be ongoing in 2018-20.

### **AMD On-line Tool Kit**

L Hoffman-La Roche AG provided an unrestricted educational grant that funded the development of develop an accessible, state of the art toolkit on AMD. This central online hub highlights the importance of improving screening processes globally to enable early diagnosis of AMD leading to prompt access to appropriate treatment. The toolkit provides detail on the various stages of AMD including, early, intermediate as well as late stage and will include specific information on Geographic Atrophy. The toolkit includes detailed information on the reality of living with these life changing conditions and will highlight the urgent need for greater understanding and support. The toolkit targets a wide and varied audience of stakeholders including the general public, at risk groups, patients and those who care for them, medical professionals and policy makers.

Vicky McGrath was hired to project manage the development of the toolkit along with the RI team and a global coalition of patients, researchers, scientific writers. Although contracted to work six months from June 2017 we are now in a position to extend the contract to allow for translation of specific aspects into six languages. The toolkit was launched on November 27<sup>th</sup> 2017.

### **EU Projects**

We are continuing to work at EU level on policy concerning research, diagnosis and access to therapies. We are gaining expertise in regulatory affairs and have made excellent connections with high level institutions and their representatives. This engagement is critical as not only does it allow us the opportunity to capacity build but our presence means the retina is seen as a progressive community. We have the opportunity to lobby directly to policy makers on our own objectives and potential partners can note our ability to engage and make a case for our community in a professional manner. This does take up time but it is useful to our membership and gives critical visibility at this stage of our development.

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These are some of the activities we are involved with:

- Working with stakeholders on policy to streamline research and healthcare in EU grant systems
- Working group to develop EU Parliament Special Interest Group on Rare Disease
- Presenting data on our work and chairing working committees at the Eurordis Round Table of Companies (ERTC)
- Ongoing work on UN Committee of NGOs for Rare Diseases
- Working with Rare Disease International
- Opened discussion on the importance of supporting grants to develop PROMs with Director General of Health at EU Commission x 2

### **ERN-EYE (European Reference Network for rare Eye Disease)**

The ERN-EYE application was coordinated by Professor Dr. Helene Dollfus (Strasbourg) and her team including Dr. Dorothee Leroux. The Core organising committee consisted of Prof. Dollfus, Dr Leroux, Prof. Eberhart Zrenner, Ms Christina Fasser (President) and Ms Avril Daly (CEO). The ERN was accredited by the European Commission at an event in Vilnius on March 9<sup>th</sup> 2017. The kick off meeting of ERN-EYE took place in Tubingen Germany on April 3<sup>rd</sup> to 5<sup>th</sup> 2017. The CEO and president are represented on appropriate sub-groups, the president on the retina group and the CEO in paediatrics as well as the communications and education group and the genetics group. The president was elected as one of two patient representatives on the overall coordinating committee. An ontology meeting to look at the classification of rare eye disease (RED) will be held in St Odile, France in October 2017 and will be attended by the president. The ERN-EYE represents excellent visibility as an area of innovation in the EU putting it in prime position for funding through EU instruments but also for transatlantic and global opportunities.

### **Projects planned**

- Potential of working with member organisations to develop funding cohorts for specific research questions (to be discussed at MC Meeting)
- Regional advocacy training programme
- Online disease specific toolkits – 1<sup>st</sup> quarter LCA
- On line toolkit on the development of therapies for retinal disease

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- Youth Programme
- Web-based Communications Programme
- Developing Voluntary Groups and Foundations
- Fundraising and Sustainability
- Managing the Retina International Network of Partners

## **Financial Report and Forecast of RI-office**

See attached document.

## **2.7 Work Plan 2017 – 2018**

See attached document.

## **2.8 Web Development Plan**

See attached document

## **Fundraising – Structure and Sustainability**

As Retina International continues to operate under EU law, the manner by which it is funded must adhere to a particular set of guidelines for the charitable sector which calls for transparency in funding. Diverse funding strategies have been developed by the RI team to focus on partnerships with, Industry, Philanthropic Foundations and increased involvement in European Union Project/Operations grants.

RI attended the Black Pear Gala in Brussels which was addressed by Avril Daly as CEO of Retina International and attended by Ms. Christina Fasser. The audience is made up Members of the European Parliament (MEPs) Ambassadors, First Ladies and heads of Biotechnology and Pharmaceutical companies in the Rare Disease Space. The Patron of the event is Princess Astrid of Belgium.

The next day the president and CEO attended an EU symposium on Access to Medicines and Orphan Drugs where the CEO spoke on behalf patients on the issue of patient reported outcomes and the 'Value' of medicine.

## **ERN Meetings**

The First organising Committee for the ERN-EYE took place on February 26<sup>th</sup> in Zurich. The larger organising committee meeting was held in

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Strasburg on April 18<sup>th</sup> and was attended by the President and CEO. From February to June a conference call was held by the organising committee to develop the ERN application on Tuesday evening at 5.30pm CET.

### **ARVO**

The president and CEO attended the ARVO meeting from May 1<sup>st</sup> to 5<sup>th</sup> 2016. This included the facilitation of the RI SMAB meeting.

### **ECRD – European Conference on Rare Diseases and Orphan Drugs**

### **Retina International World Congress – RIWC**

### **EU Retina Conference Copenhagen**

### **EU Patient Summit**

### **UN committee on Rare Diseases:**

### **Retina 2017**

### **Macular and Retina Disease Congress Seville 2017**

### **Retina International Team**

### **December, 2017**

## **Report of the CEO**

This document outlines the progress of ongoing activities undertaken by the Retina International Office

SPARK Therapeutics have funded the development of an online toolkit on genetic testing for IRDs. The toolkit is entitled RED Alert playing on the term Rare Eye Disease and taking ownership of this term for RI.

The Toolkit consists of text describing the fundamentals of genetics, the evolution of testing services, the types of tests available and where they can be accessed. There is also a section on the risk and the benefit the important issues to be considered and the importance of genetic counselling services. The final section details the way in which we can work together to advocate for better access to testing and services.

The toolkit has followed best practice and each text section is kept short and digestible but we have included in each an explanatory accompanying video clip in the hope that these will be of benefit to those who are vision impaired

A survey of patients and member organisation has allowed us to understand the areas where there was a clear lack of information and tailor the information to meet that need. The surveys have also shown up worrying trends in referrals and overreliance on research grade tests.

The text has been reviewed by three scientists and one clinician. A group of individuals with various IRDs and a group of young mothers with no knowledge of IRDs. This is to ensure that the information is useful to those who are aware of the basics and that it is not frightening or not understandable to those who are newly diagnosed.

The toolkit will be presented on beta format at EU Retina and formally launched to our members on World Retina Day. The sponsors are very happy with this timeline and have been supportive throughout the process.

### *2.2.3 AMD tool Kit*

### *2.3 EU projects*

### *2.4 ERN (European Reference Network for rare Eye Disease)*

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The ERN-EYE was launched on March 9<sup>th</sup> 2017 and the Kick off meeting in Tübingen was taking place on April 3<sup>rd</sup> – 5<sup>th</sup> 2017.

## *2.5 Projects planned*

- Regional advocate training programme
- Youth Programme
- Communications Programme
- On line toolkit on the development of therapies

## *2.6 Financial report and forecast of RI-office*

## *2.7 Work plan 2017 - 2018*