



Choroideremia

RESEARCH FOUNDATION

CRF OHIO VALLEY

PRESENTED BY THE CHOROIDEREMIA RESEARCH FOUNDATION

September 30, 2017

The Hampton Inn, Columbus, OH

MEETING AGENDA AND SCHEDULE

8:00 – 9:00am – Meeting registration, social hour. Coffee, refreshments provided.

9:00 – 9:15am – Introduction – Bob Pettipaw. Emcee & fellow CHMer

Topics – Meeting agenda. Personal history. Genetic link of CHM. Brief history of current and pending natural history studies and clinical trials. Local and national resources for CHMers. Benefits of joining the CRF.

9:15 – 9:45am – Starting the CHM Community – Jason McKinney

Topics – Starting the original CHM email support group, the importance of mutual support for those affected by rare diseases.

9:45 – 10:15am – Cory MacDonald. Co-founder & CHMer Engagement Director - CRF

Topics - CRF History/Overview, avenues to Support/Information, Fundraising.

10:15 – 10:30am – Break

10:30 – 10:55am – Siobhan Gaynor, Spark Therapeutics

Topics – Update on Spark's gene therapy trials, ID Your IRD program.

11:00 – 11:30am – Dr. Byron Lam, Bascolm-Palmer Institute, Miami

Topics – Update on Nightstarrx gene therapy trials.

11:30 – Noon – Robert Driscoll, Brett Sims & Bob Pettipaw

Topics – Gene Therapy from the patients' perspective. Open Q&A with 3 patients who have received the gene therapy surgery.

12:00 – 1:00pm - Break for lunch. Lunch provided.

1:00 – 1:30pm – Vicky Prahin, American Council for the Blind-Ohio

Topics – ACB-Ohio overview, Ohio & neighboring state resources (mobility, education, employment, independent living)

1:30 – 2:15pm – Randy Wheelock - Chief Advisor for Research and Therapy Development - CRF

Topics – The science of CHM, History and genetic link. Current and future natural history studies and clinical trials. Other treatment approaches in the pipeline, e.g. replacement therapies, pharmaceutical therapies. Early results. How to be involved in the process.

2:15 – 2:30pm – Break

2:30 – 2:45pm – Bob Pettipaw & Cory MacDonald

Topic – CHMer Engagement CARS (Contact A Retina Specialist) program): How YOU can help the CRF find more members of the CHM Family

2:45– 3:00pm – Bob & Paige Pettipaw

Topics – Genealogy and Family History resources. Family dynamics and confronting the “elephant in the room”.

3:00 – Open ended - Closing remarks and open Q&A.

For those that wish to stay a while, meet, converse and network with your fellow members of the CHM Family, the speakers will stay as long as necessary. We are all here to help each other in any way we can!

Thank You for attending the CRF Ohio Meeting! Together, we will have a Cure in Sight!