“From Bench to Bedside - The journey to creating a sight-saving drug.”

December 6th 2020, 12:00 – 15:00 CET

To register, click here.

Introduction
A series of bite-size talks by Early-Stage Researchers from the TransMed consortium, that will take us through the step-by-step process of drug development, clinical trials, and beyond. This event is ideal for those living with a Retinal Degeneration, or for those who are simply curious about learning the start-to-finish process of translational medicine, and the story of life changing research.

Schedule:
12:00 - 12:15: Opening remarks
12:15 - 12:50: Session 1
12:50 - 1:00: Break
1:00 - 1:50: Session 2
1:50 - 2:00: Break
2:00 - 2:50: Session 3
2:50 - 3:00: Closing remarks
Itinerary

Opening remarks: Chairlady Manisha Prajapati, & Christina Fasser, Former President of Retina International.

Session 1
Talk 2: “Therapeutic Challenges for Inherited Retinal Degenerations”, by Li Huang.

Session 2
Talk 4: “How and where to test drugs”, by Arianna Tolone.
Talk 5: “The journey of discovering new potential therapeutic targets in the retina.”, by Michel Rasmussen.

Session 3
Talk 11: “Last but not least: Clinical trials – Part 2.”, by Laura Lorenzo.

Closing remarks: Chairlady Anna Karpinska.
Talk Summaries:


Our eyes are our windows opening to the world. In our body, the retina is one of the most complex biological tools. The retina can convert the light into electrical signals thanks to the most sensitive cells in our body: photoreceptor cells. Understanding the photoreceptor cell’s functionality may help us to combat retinal degenerative diseases. In this talk, we will have a look at the smallest world inside of the eye, focusing on how we see the objects and then explore the magic of light in the retina.

Talk 2: “Therapeutic Challenges for Inherited Retinal Degenerations” by Li Huang.

Retinal degeneration is a type of disease for which decades ago nothing could be offered from clinicians to most people affected by it. To date, it is still a major cause of incurable vision loss and is largely untreatable. The retina, found at the back of the eye, is this “tiny” tissue contains important cells that are crucial to vision. However, there are many causes for Retinal Degenerations. In this talk, we will focus on why this makes it hard to find effective treatments, and look at some possible approaches for therapy.

In my talk, I would like to give a brief introduction of the TransMed program, which has the main aim of educating the next generation of scientists in translational medicine on eye diseases. This innovative program is ambitious to educate “translational researchers” that turn scientific output into treatment development of eye diseases.

This consortium consists of academic and non-academic partners with specific expertise covering different aspects, including basic research in disease mechanism, target definition, drug delivery system, pharmacokinetics, and drug manufacturing. All early stage researchers who engage in this project will be introduced in this talk.

Talk 4: “How and where to test drugs” by Arianna Tolone.

Before their approval, drugs need to be tested in order to verify their efficacy. The first model where drugs can be tested are cell cultures. Once the efficacy of a drug on cells has been ascertained, it’s time to switch to a model a little closer to a real retina. This is why retinal explant cultures are used. These are retinas extracted from animal models, such as mice, that are cultivated and grown. During cultivation, the drug is applied. What is special about organotypic retinal explants? They can be prepared from animals with genetic mutations similar to human ones, where the effects of the drug can be analyzed.

Retinitis pigmentosa is a common eye disease. This eye disease affects the retina, which is located in the back of the eye. In the retina, there is a layer of cells called retinal photoreceptors, which are crucial to our vision. Upon retinitis pigmentosa, the photoreceptors die by an unknown mechanism. We do know that a system called “cGMP system” plays a critical role in photoreceptor death. During photoreceptor's death, the cGMP levels increase as do some of the proteins that bind to cGMP. These proteins are called cGMP-binding proteins. Interventions with known cGMP-binding proteins have been shown to decrease the number of dying photoreceptors. This raises the possibility that known and new cGMP-binding proteins may give us a better understanding of photoreceptor death and improve therapy development. You can compare the search for cGMP-binding proteins to fishing. When fishing, you choose a rod, bait, and location depending on the fish you want to catch. When “fishing” for proteins, you use the same principles. Your method of choice is comparable to the fishing rod and bait, whereas the location is comparable to the biological sample where you choose to “fish” to catch your protein of interest.


We go to a doctor, when we face a certain discomfort or symptoms of a disease (eg. pain, fever, vomiting etc.). But how does a doctor know if we have a particular disease or what kind of treatment should be followed? The answer is through- ‘Biomarkers.’ Biomarkers are indicators of a disease. They are specific molecules found in the ‘samples’ taken from the patients. Biomarkers are crucial in every stage of drug development- from drug design to testing the drug during clinical trials. In this talk, I will focus on some biomarkers that are already in use for eye diseases.

Imagine you want to visit your family for the Christmas holiday. Depending on where you are and where your destination is, you will have several means of transportation. The usefulness of these transportation options varies case-by-case, sometimes using a car is better than train or airplane and vice-versa. Similarly, in the world of medicine, there are many “vehicles” that you can use to ensure a drug reaches its intended target, and one can be more suitable than others depending on the intended application.

These “vehicles” are called Drug Delivery Systems (DDS). Examples of DDS include liposomes, solid lipid nanoparticles, and cyclodextrin. In general, they can be designed to provide benefits such as protection from environmental conditions and improvement in cellular uptake.


Chemistry can sometimes be thought of as abstract, boring, or destructive. In reality, it is as material as the floor you stand on, as healthy as the food you eat, as fascinating as firework lights, and as powerful as every life-saving drug ever made.

In the pharmaceutical industry we use chemistry to build molecules - or synthesize drugs - which can be used to treat illness... But what does that look like? How can we know what makes a possibly sight-saving drug? And what does it take to transform this knowledge into a synthesis that can provide thousands of kilograms for millions of people? Well... let us look at one day in the life of a chemist.

Drug delivery systems facilitate administration of various drugs to patients. Broad examples include tablets, capsules, ointments, inhalations, implants, injections, etc. These delivery systems can be administered in many ways. Various factors influence the way these delivery systems are administered, e.g. properties of drug and drug formulation, and the site of action.


According to the WHO, “clinical trials are a type of research that studies new tests and treatments and evaluates their effects on human health outcomes”. Although conducting a well-designed clinical trial may appear straightforward, it is founded on rigorous methodology and governed by key ethical principles. In this talk, we provide an overview of the process of obtaining approval of a therapy, from its pre-clinical phase to post-marketing surveillance. While pre-clinical studies involve animals and explore the mechanisms of action of the drug or how it is processed after administration, clinical studies are performed on humans and are divided into 4 phases. The 3 first phases are aiming at evaluating the safety and effectiveness of the drug candidates. The fourth and last phase comes after approval, and provides additional information including risk, benefits and best use.

Sometimes, it might be hard for people to understand why it takes so long to develop a treatment, why it is so challenging and expensive, and why the vast majority of treatment candidates fail Clinical trials and are rejected.

In this talk, we touch on why Clinical trials are so important, what are the challenges associated with each phase, the main challenges it poses on drug development, and some of the risks and benefits that companies and individuals face when taking part in Clinical trials.
About TransMed

**transMed - Educating the next generation of scientists in translational medicine: Focus on eye diseases**

Medicine today experiences a gap between basic research and successful clinical translation that delays establishment of urgently needed therapies. This is very clearly so in blinding retinal degenerations (RDs), where most are yet untreatable, even with a wealth of basic and pre-clinical research data available. To address this problem, transMed proposes an innovative programme to educate “translational researchers” that focuses on the bench-to-bedside development of treatments for RD.

The transMed consortium joins four academic groups with four non-academic and SME partners, and five associated partners with intersectoral collaborations already ongoing. Each transMed partner will contribute specific critical expertise to a curriculum covering all major aspects of translational research: From basic research into disease mechanisms and target definition, to drug design and development, in vitro test systems and in vivo disease models, drug delivery systems, biomarkers, good manufacturing practice, toxicological testing and pharmacokinetics, regulatory affairs, intellectual property, all the way to clinical trials and commercialisation.

transMed builds on and integrates three relevant translational projects, which are at the early pre-clinical, late pre-clinical, and early clinical stages, respectively, to provide the project’s PhD students with the broadest possible overview. The training is completed by e-lectures, the inclusion of dedicated conferences for young researchers, a secondment and hands-on course programme from industry to academia and vice versa, permitting further insight and networking in the European biotech industry. Altogether, transMed will offer its students the opportunity to obtain a competitive PhD degree in several critical areas of biomedical research, providing for a strong employability in both the private and public sector.

About Retina International

Retina international (RI) is a global umbrella group representing the voice of 43 patient-led organisations, charities and foundations on all continents, who support the development of retinal research for unmet need. Since being established by patients affected by retinal dystrophies in 1978, RI has worked with its members, its scientific and medical advisory board (SMAB) as well as industry partners and legislators to develop policies to improve research infrastructures and the delivery of health and support services for people living with retinal dystrophies across the globe.

It is also focused on improving access to early detection services that can lead to the timely diagnosis of retinal disease and access to appropriate interventions to save sight. This includes systematic screening of conditions that affect the aging eye such as Age-related Macular Degeneration (AMD) and Diabetes-related Eye Disease (DED), while also advocating for equitable access to genetic testing services for Inherited Retinal Diseases (IRDs).

The overarching goal of RI remains the promotion of innovation that has the potential to change the lives of those who live with retinal disease. In order to ensure success, we believe that patient engagement is critical at all stages of the research journey, from concept to delivery.

RI is recognised as a leader in global patient advocacy and has always promoted a multi-stakeholder approach to its work. RI believes that now is the time for the organisation to apply a process of structured collaboration with patients, industry and regulators in ensuring faster progress in healthcare innovation to address the unmet need of the retina community.

www.retina-international.org