APV Focus Group Drug Delivery

Combining Science & Technology to Create Advanced Drug Delivery Systems

International Association for Pharmaceutical Technology

Newsletter ISSUE 1/2023 - August

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Suggest a meeting to be announced!

DRUG DELIVERY PRODUCTS

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Provided by Dr. Louise Rosemayr-Templeton

Syfovre™

On 17 February of this year the US FDA approved Syfovre™ Injection, the first and as yet only approved therapy for geographic atrophy (GA) caused by age-related macular degeneration (AMD) [1, 2]. It is licensed for the treatment of GA patients with or without sub foveal disease. It was developed by Apellis Pharmaceuticals Inc. (MA, USA) and contains pegcetacoplan, a pegylated complement protein 3 (C3) inhibitor. It has been shown to be effective when dosed every 25 days to 60 days, which enables some flexibility in dosing regimens.

GA is an advanced form of AMD and affects 5 million patients globally with around 1 million in the US alone [1, 3]. It is a major cause of blindness in the elderly and leads to loss of independence and reduced quality of life. Patient numbers are likely to increase dramatically in the future due to aging populations and increased life expectancy with a predicted 18.7 million sufferers by 2040.

In general AMD affects both eyes, and vision loss is considered permanent. Genetics, age, environmental factors and oxidative stress are considered risk factors for developing this condition. In early stages of the disease extracellular deposits are visible as dots at the back of the retina. These deposits plus other factors lead to inflammation. Defects in the regulation of the complement system are thought to have a key role in its development promoting inflammation and cell death, which leads to a gradual reduction in retinal pigment epithelium photoreceptors, and associated choriocapillaris. Typically, geographic atrophy initially affects the areas outside the fovea first leading to a loss of peripheral vision under conditions of poor light. The loss of vision progressively worsens until the fovea is involved with

loss of central vision leading ultimately to blindness. The median time between the start of geographic atrophy and the beginning of central vision loss is 2.5 years.

Pegcetacoplan is a 43.5 kDa symmetrical pegylated protein consisting of two pentadecapeptides bound to the same 40 kDa polyethylene glycol molecule. Each peptide portion of pegcetacoplan has the same amino acid residue composition including at position 4, a 1-methyl-L-tryptophan (Trp(Me)) residue and in position 14, an amino (ethoxyethoxy) acetic acid (AEEA). Syfovre™ is marketed as a single vial containing 0.1 mL of a sterile solution of 150mg/mL pegcetacoplan. The dose is 0.1 mL (15 mg) in each affected eye every 25 to 60 days with a reduced incidence of neovascular AMD occurring in clinical trials when the injection was administered every other month. Other excipients include trehalose dihydrate, glacial acetic acid, sodium acetate trihydrate and water for injection. If necessary, pH adjustment to pH 5.0 is achieved using sodium hydroxide and/or additional glacial acetic acid.

Pegcetacoplan inhibits complement activation by binding to C3 of the complement cascade and its activation fragment C3b. Binding prevents C3 cleavage and its further amplification through C3b. C3 cleavage is a common step in all three complement activation pathways (classical, lectin and alternative). Inhibition of complement activation hinders inflammation and cell death mediated through downstream effectors of the complement system.

Two Phase 3 trials (OAKS and DERBY) were conducted to investigate the safety and efficacy of Syfovre™ in a total of 1258 patients with GA (atrophic, non-exudate age-related macular degeneration) secondary to AMD with/without sub foveal involvement. Participants between the ages 60 to 100 years (mean 78.7 years) were randomised to receive either Syfovre™ (839 patients) or a sham injection (419 patients) for 24 months. In each study randomisation assigned patients in a 2:2:1:1 ratio to each of four cohorts: one received Syfovre™ every month; the second were administered the intraviral injection every other month; the third cohort were given a sham injection monthly and the fourth the sham injection every second month. At the start of the OAK study the mean total area of GA lesions was 8.23 mm^2 (standard deviation 3.83 mm^2), while for the DERBY study it was 8.29 mm^2 (standard deviation 4.11 mm^2).

31% of participants administered Syfovre™ monthly in the OAK trial and 21% of those given the injection every second month stopped treatment prematurely prior to Month 24. This compared to 25% of the patients in the sham injection cohorts. In comparison in DERBY, the discontinuation figures were 29% (Syfovre™ monthly), 22% (Syfovre™ bimonthly) and 21% (sham injection).

Both studies showed that Syfovre™ treatment resulted in a reduction in the mean rate of GA lesion growth compared with sham as measured by fundus autofluorescence. At the completion of the OAK study there was a 21.9% and 18.1% reduction in the rate of GA lesion area growth compared with baseline following Syfovre™ monthly and bimonthly treatment respectively compared with sham injection. In the DERBY study the % reduction in lesion growth was 18.1% and 17.4% for the respective administration regimens. The treatment benefit increased with time with the maximal reduction in lesion growth compared with sham injection being 36% in the DERBY study for monthly Syfovre™ administration if only the 19-24-month period was considered.

Side-effects experienced in Syfovre™ cohorts in the Phase 3 trials at an incidence of ≥5% were ocular discomfort, neovascular AMD, vitreous floaters, and conjunctival haemorrhage. Anti-pegcetacoplan peptide antibodies were detected in the OAKS and DERBY studies at an incidence of 4% (18/415 evaluable patients) and 2.5% (10/404 evaluable patients), respectively.

The product is undergoing regulatory review by the European Medicines Agency with approval anticipated in early 2024 [1]. Sales of this potential blockbuster drug are predicted to reach USD 2.6 billion by 2028 [4].

Hemgenix®

On 20 Feb 2023 CSL Ltd (Marburg, Germany) announced that Hemgenix® (etranacogene dezaparvovec) had been awarded a conditional marketing authorisation by the European Commission to treat severe and moderately severe haemophilia B in adults (inherited Factor IX deficiency) who do not produce Factor IX inhibitors [5]. These inhibitors are autoimmune antibodies against Factor IX medicines and, as a result, reduce their effectiveness. Approval followed a positive opinion on the product from the European Medicines Agency (EMA)'s Committee for Advanced Therapies (CAT) and the Committee for Medicinal Products for Human Use (CHMP) in mid-December 2022 [6]. Hemgenix®, which is administered as a one-dose intravenous infusion, is the first gene therapy product to be approved for haemophilia B in the European Union and the European Economic Area.

Haemophilia B is a serious, rare, genetic disease, estimated to affect 1 baby boy per 20,000 to 50,000 male births in Europe [6, 7]. Mutations in the gene for Factor IX results in the absence or below normal levels of circulating Factor IX. Patients suffering from this condition are susceptible to bleeding in joints, muscles and internal organs which in turn leads to pain, swelling and tissue damage. Until the approval of Hemgenix®, the only treatment option for Haemophilia B sufferers were regular infusions of Factor IX to replace or boost the levels of this clotting factor.

Hemgenix® (also known as CSL222 and previously as AMT-061) contains adeno-associated virus five (AAV5) as the vector for the delivery of the Padua gene variant (variant R338L) of Factor IX to hepatocytes [8]. This naturally occurring variant of the gene for Factor IX results in proteins which have five to eight-fold the activity of normal Factor IX [5]. Expression is controlled by the liver-specific promotor, LP1. Manufacture of etranacogene dezaparvovec is carried out in insect cells using recombinant DNA techniques. Each vial contains 10 mL of a concentrate containing 1 x 10^{13} genome copies (gc) of the Padua gene variant of Factor IX per ml. The concentrate is diluted with 0.9%w/v saline prior to intravenous infusion. The dose is 2 x 10^{13} (gc) per kg body weight and is administered only once.

The product was developed and taken into clinical trials by uniQure NV (Amsterdam, Netherlands). The company has the sole global rights to use the AAV5 vector for therapeutic products for brain and liver disease. uniQure's data

suggest that this vector may not be so susceptible to removal by anti- AAV antibodies raised as a result of previous exposure to AAV through dosing or naturally acquiring infections [9]. In June 2020 CSL Behring gained exclusive development and commercialization rights to Hemgenix® in a deal potentially worth over USD 2 billion to uniQure [10].

The conditional approval in the EU and EEA was mainly based on the results of the pivotal HOPE-B Phase III trial which is the biggest ongoing gene therapy study in haemophilia B to date. In this open-label, single arm trial 54 adult sufferers of moderately severe to severe haemophilia B first completed an observational ≥6-month period during which they received their standard-of-care Factor IX replacement therapy. The purpose of this pre-treatment period was to establish an Annual Bleeding Rate (ABR). Following this, they received a single dose of Hemgenix® of $2x10^{13}$ gc/kg by intravenous infusion. The trial's primary end-point was 52-week ABR from Month 7 to Month 18 following infusion. This time-frame was selected to allow stable gene expression to be established in the first 6 months post-treatment.

The 18-month post-infusion results showed that Hemgenix® administration produced a mean Factor IX activity of 36.9 IU/dL, while at 24 months it was 36.7 IU/dL demonstrating that the increase in circulating Factor IX is maintained 18 months following infusion. The adjusted ABR from Month 7 to Month 18 was reduced by 64% ($p= 0.0002$) compared with the first six-month observational period (ABR decreased from 4.19 to 1.51). In addition, all Factor IX treated bleeds decreased by 77% from 3.83 over 6 months to 0.83 between Month 7 and Month 18 ($p < 0.0001$). Treatment with Hemgenix® also caused a major reduction in regular, routine Factor IX replacement therapy with 52 out of the 54 patients enrolled in the study no longer requiring prophylaxis therapy between Month 7 and Month 24 (96.3%). The mean reduction in Factor IX administration was significant and amounted to 248,392.6 IU/year/patient (96.52%; 1-sided $p< 0.0001$) between Month 7 to 24 compared to that required during the 6-month observational period. The study results to date demonstrate the potential of Hemgenix® to replace routine prophylactic Factor IX administration by enabling the patient's liver to produce clinically significant amounts of Factor IX activity in a durable way over the time-period studied.

No serious side-effects were attributed to treatment. Liver damage, as indicated by increased circulating levels of liver transaminases, was commonly observed. However, only 23% of patients experienced symptoms as a result. This is a known side-effect of AAV gene therapies which is caused by an immune reaction and can be resolved by use of corticosteroids [8, 11]. Other common adverse effects reported included increased bilirubin and blood creatine phosphatase, nausea, headache and flu-like symptoms. There was also no significant clinical change in the levels of AAV5 neutralising antibodies at baseline and Factor IX activity.

Two serious adverse events occurred during the trial: an elderly participant died from urosepsis and cardiogenic shock 65 weeks following the Hemgenix® infusion and another developed hepatocellular cancer. However, following analysis, both these events were considered unrelated to treatment. Patients, who took part in the trial, will be monitored for a number of years to assess the long-term safety and efficacy of the treatment.

Hemgenix® was approved by the FDA in November 2022 and is currently considered the most expensive drug in the world with a price tag of $3.5 million per one-time treatment. However, CSL has estimated its use will result in US healthcare cost savings of $5 million to $5.8 million per patient based on the reduction or removal of the need for chronic Factor IX replacement therapy [12].

References and Further Information

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ACTHERA THERAPEUTICS LTD (BASEL, SWITZERLAND)

Acthera Therapeutics is a Swiss biotech startup, developing a unique and innovative type of liposomes, for the targeted and controlled delivery of drugs. Based on a patented synthetic lipid, they produce Hard-Shelled Liposomes (HSL) that are stiff and have a dodecahedric shape, with faces and edges, a unique characteristic among liposomes. This gives the HSL a strong differentiator: they are mechano-responsive. When submitted to appropriate mechanical stress, HSL release their payload, and this unique ability allows the controlled and targeted delivery of drugs. Thus, the technology is an excellent is the ideal way to maximize the therapeutic potential of multiple drugs, increasing efficacy by better targeting the drug to its target and reducing unwanted systemic effects.

The combination of HSL with various payloads and specific release triggers opens a large field of therapeutic opportunities in many clinical indications. Multiple payloads can be encapsulated, from small molecules, to RNA and peptides. The encapsulation of the therapeutic agents is a protection against metabolism or excretion, until the drug is locally released to its cellular or tissular/organ target, optimizing the PK/PD parameters.

Various mechanical triggers can activate the release of the HSL's payload. High shear stress associated with a pathological vascular occlusion is an internal trigger that can be used to deliver active drugs at the site of an acute thrombosis, in acute myocardial infarction or acute ischemic stroke. The company is therefore currently focusing on developing new thrombolytic drugs, with an increased efficacy and efficiency, thanks to a controlled and targeted delivery at the site of the thrombosis, triggered by the associated shear stress.

External triggers, such as ultrasound or laser light, applied to target specific body locations or organs can have the same triggering effect as shear stress. In the future, the HSL-payload-trigger combination will be a unique platform to deliver drugs WHERE and WHEN they are needed, from cardiovascular to oncologic diseases. It will increase the therapeutic effectiveness of many drugs, whose use is still limited by their unfavourable efficacy: safety profile for the benefit of patients.

Fact sheet:

Founded:	2019
Location:	Basel, Switzerland
Ownership:	Privately funded
Employees:	9
Key technology:	Hard-Shelled Liposomes (HSL) Hard Shelled Liposomes (HSL) are made from a proprietary synthetic lipid, which gives the HSL a unique stiffness and shape, making them mechano-responsive. They release their cargo drug when submitted to an appropriate level of mechanical stress, which can be generated by various triggers (internal or external). This characteristic is used to target and control the delivery of drugs at the site of the disease, to increase the efficacy and safety of therapies. Various therapeutic applications are possible, but the lead indication is the improvement of thrombolytic therapies in major acute cardiovascular events, such as acute myocardial infarction (AMI) and acute ischemic stroke (AIS).
Products:	The HSL are a platform for the targeted and controlled delivery of drugs, and our products will be a combination of HSL and their cargo drug (API, active pharmaceutical ingredients). Many APIs can be encapsulated, from small molecules to nucleic acids and proteins, but the most promising are those with a narrow therapeutic index or a delivery issue. To develop the full potential of the HSL-API platform, its combination with a trigger generating mechanical stress is necessary. Currently, they are using an internal trigger, the increased shear stress associated with the acute thrombotic occlusion of a blood vessel, to enhance the efficacy and safety of thrombolytic drugs indicated in AIS and AMI.
Development status:	They are currently in the preclinical development phase, conducting animal model experiments, to obtain the proof-of-mechanism and proof-of-concept for our HSL-API lead indication. At the

	same time, they are already planning and starting manufacturing up-scaling activities, as well as IND-enabling studies.
Partnerships:	InnoSuisse Support
Website:	https://acthera-therapeutics.com/biotechnology-company/executive-team
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DRUG DELIVERY PEOPLE

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Provided by Dr. Louise Rosemayr-Templeton

This issue of the Newsletter features Dr. Maria Marlow, Associate Professor in Formulation Science and Pharmaceutical Materials at the School of Pharmacy, University of Nottingham, UK. Maria is a pharmacist with over 30 years of experience in drug delivery and product development in both industry and academia. She completed her undergraduate studies in Pharmacy at the University of Manchester, UK and attained her PhD on “Protein adsorption to colloid carriers” from the University of Nottingham, UK in 1991. Her research interests then broadened into tissue engineering as a post-doctoral scientist at the Massachusetts Institute of Technology (1991-1992) where she studied the interaction of osteoblasts with biodegradable polymers.

Following her return to the UK, Maria worked for 18 years in the pharmaceutical industry for companies such as Fisons, Astra and then AstraZeneca in various scientific and managerial roles including leading multi-disciplinary teams in product development and early development. Her main scientific focus was on inhaled and intravenous dosage forms, as well as formulations for poorly soluble compounds and suspension formulations. Her achievements in Industry include, a formulation patent for AstraZeneca’s Symbicort product, and a new global process for suspension development within the company.

Following AstraZeneca’s closure of the Loughborough site and with two young sons at school in Nottingham, Maria continued to pursue her love of drug delivery research by securing the first Gunn and Carter Senior Fellowship at De Montfort University, Leicester, UK working with Professor Geoff Smith on developing methodologies for studying bio-relevant physical form changes of drug products using terahertz spectroscopy and pulsed imaging. In 2012, she was appointed as Associate Professor in Formulation Science and Pharmaceutical Materials in the School of Pharmacy at Nottingham. She is currently training director of the Engineering and Physical Sciences Research Council (EPSRC) Centre for Doctoral Training in Transformative Pharmaceutical Technologies and supervises 4 PhD students and co-supervises 4 others.

Maria’s research interests at Nottingham are focused on developing drug delivery systems, in collaboration with clinicians that address an unmet clinical need. Her current research projects include the development of drug delivery systems using injectable hydrogels (including supramolecular hydrogels), nanoparticles and microneedles to deliver small molecules, proteins or peptides for the treatment of cancer (skin, ovarian and brain tumors), HIV and pain. The research involves the physicochemical characterisation of these delivery systems to allow the optimisation/prediction of their in vivo performance. She has published > 40 research articles in a range of scientific journals, including drug delivery and biomaterial readerships.

FEATURED ARTICLE

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Provided by Dr.Thierry Fumeaux and Dr. Karsten Cremer

ACTHERA THERAPEUTICS: AN INTERVIEW WITH DR. THIERRY FUMEAUX, CEO

What are the major challenges you are facing in the development of your nanotechnology?

Acthera Therapeutics is developing a nanotechnology-based platform, for the targeted and controlled delivery of drugs, in order to improve the efficacy-safety balance of drugs with a narrow therapeutic index. To accomplish this mission, we are building drug products around our Hard-Shelled Liposomes (HSL), an innovative and unique type of

mechano-responsive nanoparticles. From the beginning, the team was aware of the complexity of the task and was prepared to face multiple challenges and to do everything that was needed to bring the 1st product to the patients (and the market). The complexity of such a project is related to the development of a platform combining three elements: the HSL, an active pharmaceutical ingredient (API), and a trigger for the release. Each element brings its own set of challenges, and the key to success is to always choose the lowest possible level of complexity. However, you have to understand in detail every specific feature of the platform, from a very good understanding of the biochemical characteristics of the HSL, their interaction with physical forces leading to cargo release, to the optimization of the production of the lipid and API and their combination in the HSL-API product. The regulatory aspects and authorization process for a complex biological product are also challenging and probably more complex than for a small molecule or even a simple biological product. Eventually, it is a subtle but essential part of the game to convince our partners, investors, shareholders, or associated companies, of the great potential of future development of the HSL-based platform, while clearly defining the steps to be taken over time. This is in particular the case for the choice of the initial clinical indication enabling the demonstration of the safety of the HSL and proving the therapeutic concept.

Why did you choose acute cardiovascular events as your lead clinical indication?

As mentioned, this choice was a major strategic decision. The main consideration was the key differentiator of the HSL, namely their mechano-responsiveness, which had to be transformed into a competitive advantage and define our unique selling proposition. Based on this, we had to select a lead clinical indication and primary compound, associated with a potential physical trigger for the targeted release, with the objective to keep the level of complexity as low as possible, and thus mitigating the risk of failure during this initial development. We therefore defined a series of criteria to guide the best possible choice among the hundreds of potential combinations. Some were quite obvious, such as the selection of an API that was already on the market but off-patent, with a well-defined efficacy and safety profile leaving space for improvement, while decreasing the risk inherent to the drug itself. However, we were also looking for an indication for which the HSL and the API could ‘work’ in a single body compartment, solving the issue of tissue distribution and cellular interactions. In addition, the selection of a trigger that would not need to be specifically developed for use in combination with the HSL in the clinic was also seen as essential. For these reasons, a single intravenous injection in an acute disease was considered less risky than dealing with other administration routes in a chronic condition needing repeated doses. When you consider all these factors, it immediately becomes evident that acute cardiovascular events, such as acute ischemic stroke and acute myocardial infarction, are the ideal clinical conditions to address, and that clot-lysing drugs are the best possible API to associate to the HSL. This would enable the use of an internal pathological physical trigger, namely the massively increased shear forces generated when a blood vessel is obstructed. Moreover, due to an unfavorable efficacy:safety balance, there is a clear unmet medical need in the management of these events, which are the number one cause of death around the world, killing millions and leaving even more patients with disability.

How do you finance such a project?

As mentioned, this is a project with a high potential of application in multiple clinical indications. So it is easy to make people dream. However, before that you have to convince your investing partners that you can master the complexity of the development and that you have precisely and adequately defined our financial needs for the successive steps needed to bring the product to the market and generate a return on investment. The global cost of such a development is generally underestimated, particularly those related to the manufacturing and the regulatory process. We have therefore anticipated as much as possible the cost of all operations that will be needed along the pre-clinical and early clinical development phases. We adopted a lean approach, based on the principle to target the ’MVE’, the minimal viable experiments allowing for systematic progress and reducing the risk of failure later on. Since the incorporation of the company in 2019, our activity has been was financially supported by angel investors and non-dilutive funds (grants), complemented by a Covid-19 loan. This was obtained against the background of a war and economic crisis following the pandemic… Surely not the ideal timing but one we think is a good test of resistance for the company. For the financing rounds to come, we will continue to rely on a hybrid financing strategy, combining funds from our current shareholders and investors, new private investors, non-dilutive funds, venture capital funds, and co-development or licensing agreements. This mix allows us a certain flexibility in our strategy, adapted to the evolution of our development conditions. Clearly, we want to avoid opening a round of financing every 6 months, but we have to take reality into account and adapt.

Where do you see Acthera in 5 years’ time?

The common vision of the team is that Acthera will be a company enabling a significant improvement in the efficiency (more efficacy, more safety) of many pharmacological therapies, in various diseases, and for a large number of patients. We want to have a real impact! To realize this vision, Acthera could either be an established pharmaceutical company, marketing its own drug-products, or the partner of other companies, contributing under licensing or co-development deals to bring better therapies to patients in need. We are all working hard to make this a reality.

ABOUT THE FOCUS GROUP

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The APV Drug Delivery Focus Group (APV DD) is a section of the APV (Arbeitsgemeinschaft für Pharmazeutische Verfahrenstechnik e.V. / International Association for Pharmaceutical Technology), a major European society for those sharing a professional interest in pharmaceutical sciences. The Focus Group was established in 2003 in response to the increasing importance of drug delivery within modern pharmaceutics.

COMBINING SCIENCE AND TECHNOLOGY TO CREATE ADVANCED DRUG DELIVERY SYSTEMS

OUR MISSION STATEMENT:

Modern drug delivery research and development is a truly multidisciplinary approach and must combine all relevant scientific, technical, medical and regulatory aspects required for the design, preparation, testing, manufacturing and registration of drug delivery systems and their components. It is the mission of the APV Drug Delivery Working Group to foster and promote all aspects of research and development required to transform drug molecules into safe, applicable and acceptable drug delivery systems, which provide therapeutic benefit, convenience to the patient and improve patient compliance.

Our mission includes in particular the following tasks:

- Thoroughly understanding the physical-chemical and biopharmaceutical properties of the drug substance to be delivered and the components of the drug delivery system
- Understanding the biological barriers and the interactions of the drug molecule and its delivery system with the biological environment and the biological target including PK/PD and PK/safety relationships
- Research on excipients, materials and technologies required for the design, preparation and manufacturing of drug delivery systems for a selected route of administration
- Development and understanding of methods for in vitro and in vivo evaluation of drug delivery systems and their components
- Knowledge of regulatory requirements for clinical testing, manufacturing and registration of drug delivery systems

All disciplines relevant to the above-mentioned areas of drug delivery R&D are invited to contribute to the APV Drug Delivery Group:

Pharmaceutics, Biopharmaceutics, Analytics, Biology, Physical Chemistry, Biochemistry, Physics, Engineering Sciences, Nano Technology, Material Sciences, Polymer Science, Toxicology, Drug Safety, Clinical Research, Drug Regulatory Affairs, etc.

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