TOPRA ANNUAL SYMPOSIUM
STOCKHOLM, SWEDEN

In partnership with the Swedish Medical Products Agency

Time of transformation: Implementing international regulatory change

A regulatory update in a day for Small to Medium-sized Enterprises

3 October 2018

Wednesday 3 October 2018	
08.30	**Registration and Exhibition**
09.00	**Introduction to SME Day**
	Margareth Jorvid, Methra Uppsala AB/LSM Group, Sweden

Following introduction to the day, participants choose one of the following morning tracks:

	ATMP	Human	Veterinary
09.15-10.45	Introduction to ATMP	The Regulatory role in Early Access Schemes to Patients	Antimicrobial resistance
11.15-12.45	ATMPs (Cell- and Gene Therapies, the future?)	Clinical Trial Regulation	Advanced therapies
	Networking and light lunch in the Exhibition Hall		
13.30-15.00	SME Session This session will give small and medium-sized enterprises (SMEs) the opportunity to understand regulatory challenges specific to them. Support by the SME Office at the European Medicines Agency (EMA) will be explored and experiences by SME companies shared.		

Introduction to Advanced Therapy Medicinal Products (ATMPs) **Leader:** Jayne Hunt - Associate Director, Regulatory Affairs, Boyd Consultants Ltd, UK	
ATMPs are defined as gene therapies, somatic cell therapies and tissue engineered products. Since the introduction of the ATMP regulation (EC) 1394/2007 there are a minimal number of ATMPs that are authorised for use. There is a recognition of the specific needs of innovators developing these products and support has been provided with respect to fees and access to scientific advice. In this session we plan to provide an introduction to ATMPs and their associated regulations as background to session 9 on ATMPs. We will use examples of products that are authorised (where available) to demonstrate the types of ATMPs: cell based therapies CAR-T: gene therapies and more; opportunities for interaction with EMA and some of the considerations during development and post authorisation that are specific to ATMPs.	
Chair: Jayne Hunt - Associate Director, Regulatory Affairs, Boyd Consultants Ltd, UK	
09.15	**Introduction**
09.20	**Topic**
	Speaker TBA
	Part 1: Legal definition Differentiation between a biologic and an ATMP Overview of the regulations and directives CAT role Part 2: Pharmaceutical development roadmap From small molecules to biologics and ATMPs including CMC controls and regulatory requirement Part 3: Examples Cell based inc CAR-T / gene therapy / RNA / Fusion proteins / other Part 4: Specific considerations Shedding / Long term follow-up / Traceability / GMO based ATMPs issues and regulations Part 5: Support for all applicants / SMEs Is my product an ATMP - Certification / Scientific advice / Fees / SME office
	Panel discussion moderated by chair – The speakers will take questions from the floor.
10.45	**Break**

ATMPs (Cell- and Gene Therapies, the future?) **Leader: Vera Franzen – Sweden**	
After 10 years with the ATMP Regulation, there are only a handful of approved products on the market. With a great number of products in the pipeline there are still challenges to overcome, especially for SMEs and Academia. *In this session we will hear about plans and activities to facilitate for developers of ATMPs.*	
Chair: Vera Franzen – Sweden	
11.15	**Introduction**
11.20	**Commission perspective** or, alternatively **EMA perspective**
	Speaker TBA
	• Impact of the ATMP Regulation on advanced therapies – Report of 2014 • The Hospital Exemption • Joint Action Plan
11.40	**The CAT – ongoing activities**
	Speaker TBA
	• Clinical trials on ATMPs • Strengthened guidance on follow-up and risk management for ATMP developers • New guideline on IMPs
12.00	**A SME regulatory ATMP case study**
	Margareth Jorvid - *Head of Regulatory Affairs and QA, Immunicum AB, Sweden*
	• ATMP classification • Scientific Advice (National, EMA and FDA) • EMA ATMP Certification
12.20	**Panel discussion moderated by chair –** The speakers will take questions from the floor.
12.45	**Break**
	Networking and light lunch in the Exhibition Hall

The Regulatory role in Early Access Schemes to Patients **Leader: João Duarte, Associate Director, Europe Regulatory Policy & Intelligence, Takeda, UK**	
Patient access to medicines remains a fundamental objective to all the stakeholders involved in medicines regulation. Even though many challenges such as affordability and pricing of new medicines remain extremely cross-disciplinary, early access schemes (EAS) remain an area where the regulatory professional plays a critical role in ensuring that early patient access is balanced with benefits and risks of innovation. But what are early access schemes and how do these link to regulatory strategies in Europe? This session will shed some light to the current mapping of available early access schemes in EU Member States and how these play an important part in the voyage between medicines regulation and patient access.	
Chair: Joao Duarte – Takeda, UK	
09.15	**Introduction**
09.25	**A NCA perspective on Early Access Schemes**
	Speaker TBA
	Example and how it fits within the European picture of EAS and EMA's Article 83. • Particularities and challenges in of the LP and CUP in Sweden. NCA views on how to enhance regulatory collaboration on the matter.
09.45	**An industry perspective on how EAS supported early access to innovation**
	Speaker TBA
	• Case studies from a regulatory perspective on how industry manages EAS throughout Europe in a single development program. • Challenges found in the EAS diversity landscape in the EU. Thoughts on the role of EAS in ensuring patient access to innovation.
10.05	**How EAS can support timely access to patients in Europe: a patient's view**
	Speaker TBA
	• Examples from patients that took part in EAS (in Sweden or elsewhere) and how the information about the scheme was communicated. • Patient experience throughout the scheme and their advocacy views on patient access. Thoughts on what needs to be optimised at European level to ensure further access.
10.25	**Panel discussion moderated by chair** – The speakers will take questions from the floor.
10.45	**Break**

Clinical Trial Regulation **Leader: Susan Bhatti, Merck KGaA, Germany**	
In 2018 a notice was published in Official EU Journal that the EU clinical trial portal and database are fully functional and therefore the CT Regulation (EU) No 536/2014 will become applicable from xx xxx 2018. After a transitional period of 1 year it will be mandatory to use the portal and database for all new clinical trials in the EU, and after 3 years all trials must be transferred. This session will share the hands-on insights of testers from industry, Agencies and Ethics Committees on the portal and database functionality, as well as experience gathered by the Swedish MPA and Ethics Committees with their national CTR pilot. We will discuss transition guidance documents and the challenges of practical implementation of the legislation.	
Chair: Susan Bhatti - Merck KGaA, Germany	
11.15	**Introduction**
11.20	**CT portal update**
	Speaker TBA
	• Update on portal "go-live" and insights from an industry "on-site" tester on the CT portal and user friendliness
11.40	**MPA & EC – Swedish pilot**
	Speaker TBA
	• Update from a Health Authority / EC perspective on CT portal and agency readiness– MPA pilot -MPA & EC
12.00	**Transition guidance & CTR implementation**
	Speaker TBA
	• Update from EMA
12.20	**Panel discussion moderated by chair** – The speakers will take questions from the floor.
12.45	**Break**
	Networking and light lunch in the Exhibition Hall

Antimicrobial resistance	
Chair: **Anja Holm – Central Vet Pharma consultancy, Denmark**	
09.15	**Topic title:** Update from dose optimisation project
	Speaker name, position, organisation, country
	• Antimicrobial resistance – impact for product availability combined with update from dose optimisation project. • SPC harmonisation exercise running now.: pre-exercise of new reg. intention**.** Development and consequences.
09.35	• **EU COM mandate** (given July 2017) for AMEG advice: The mandate relates to updating the categorisation of antimicrobials and development of an <u>early hazard characterisation</u>, as needed.
09.55	• **A Major objective of new regulation is to** control AMR – summary of these legal proposals. Alternatives to AMs – how does the new reg. cater for this development.
10.15	**Questions and discussion**
	The speakers will take questions from the floor – this is your opportunity to explore the topics more fully.
10.45	**Break**

Advanced therapies	
Introductory statement……	
Chair: Beat Lohr - IDT Biologika, Germany	
11.15	**Industry perspective of SME**
	Speaker name, position, organisation, country
	• issues Are the SME initiatives useful?
11.35	**Novel therapies: update from EMA on challenges/development of advanced therapies**
	Speaker TBA
	• Other Innov. Topics also: ITF survey, Sc.Adv., Vet Innov.Day. • Promote innovation and use of new approaches in the development of novel veterinary medicines
11.55	**Future regulation: how to accommodate novel vet med + new science?**
	Speaker TBA
	• Biologicals, MRLs for novel adjuvants etc • Classification and dossier structure of novel therapy products.
12.15	**Questions and discussion**
	The speakers will take questions from the floor – this is your opportunity to explore the topics more fully.
12.45	**Break**
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SME Session **Leader: Margareth Jorvid – Methra Uppsala AB/LSM Group, Sweden**	
This session will give small and medium-sized enterprises (SMEs) the opportunity to understand regulatory challenges specific to them. Support by the SME Office at the European Medicines Agency (EMA) will be explored and experiences by SME companies shared.	
Chair: Margareth Jorvid – *Methra Uppsala AB/LSM Group, Sweden*	
13.30	**Introduction by Chair**
13.40	**SME Office at the EMA**
	Speaker TBA
	• Activities of the Agency's SME Office • Experience of EMA scientific advice process and centralised procedure • Support to SMEs
14.10	**Experience of a Swedish SME**
	Speaker TBA
	• SME challenges • Experience of drug development • Regulatory and administrative assistance • Incentives and support
14.30	**Panel Discussion** Speakers will take questions from the floor – this is your opportunity to explore topics more fully on issues of interest to you.
15.00	**Close of SME Day**