

A microscopic image showing numerous cells. Some cells are brightly blue, while others have a distinct orange or yellow core. The background is dark, making the fluorescent cells stand out.

ATMPs From idea to patient

Setting the scene

Bart Van Acker

Introduction

- **Founder of the QBD and YEOTY 2019**
- **Consulting & Solution provider in QA, Validation, Regulatory & PM**
- **190 employees and offices in 7 countries**
- **Two focal points: ATMPs and Medical devices**
- **Solutions for ATMPs companies:**
 1. **Cell by Design**
Software platform & methodology for process development for ATMPs
 2. **ATMPKick**
MES for ATMPs (developed with  **neuroplast**)
STEM CELL TECHNOLOGY
 3. **QualityKick**
Cloud based and validated Quality Management system (SAAS)

ATMPKick™: the efficient EBR system for ATMPs

Advanced Therapy Medicinal Products (ATMPs) offer great new opportunities for the treatment of patients and their diseases or injuries. In ATMP laboratories and cleanrooms all over the world, experts work on the manufacturing and quality control of these medicines, based on genes, viruses or cells. Efficient management of workflow plays a central role in these laboratories and cleanrooms. That's why QBD developed ATMPKick™ – an Electronic Batch Recording (EBR) system specifically designed for ATMPs.

Although the workflow around ATMPs is getting more and more complex, many ATMP companies still use cumbersome Excel files, spreadsheets, MS Word and email, to manage the complex workflow during their manufacturing and quality control processes. Often, these systems are prone to problems. Employees miss out on critical data, have problems with data integrity, can't validate documents or even have problems keeping an overview of documents for review or approval. The result is a high level of operational risk. You need a way to keep managing the paper-based system, you need to investigate and correct during batch processing that is wrong conditions and – if worst comes to worst – whole batches must be scrapped.

Meet ATMPKick™ – the EBR system for ATMPs
ATMPKick™ is a web-based system specifically designed for ATMPs. It enables you to manage production workflow and quality control during the ATMP manufacturing process, and the required cleanroom under-regulatory rules. The system's core features include the management of genes, viruses, nuclei, fractions and cleanrooms. In addition, users are able to create, manage and validate their own specific batch records – including audit trail – thanks to a user-friendly point-and-click interface.

The advantages
With ATMPKick™, you can digitally and automatically ATMP workflow and are able to work virtually with multiple cleanrooms, hospitals and operators. Other advantages include:
• An easily accessible web-based solution
• Scalable design for ATMPs, allowing unlimited follow-up and release of multiple, personalized and personalized production runs
• Customizable for different types of manufacturing processes
• Possibilities for dynamic checklist creation and batch runs
• Cloud-based system with backup and disaster recovery and full data
• Management of different sites and tasks to different people
• Scalability for integration with your systems
• Very easy to validate, as validation was a key factor in its development
• Developed according to FDA 21 CFR Part 312 guidelines

Pieter de Meester, CEO of Neuroplast, the Dutch medical biotech company that specializes in neuro-regenerative medicine.

"We specialize in regenerative medicine, focused on bringing high qualified autologous stem cell products to the global market environment in short track programmes. Speed and efficiency are very important in this matter. ATMPKick™ is the ideal solution to efficiently manage our production workflow and quality control. Together with the QBD Group, we developed a customized version, tailored to our specific working methods and needs. Since we work via lean principles, we are constantly extending and optimizing the solution, while we already disposed of a very useful IT system with all our functionalities. As a full service partner in validation and quality management, QBD also helps us validate the solution, so we are sure we comply with all regulations."



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QBD is also active in the Netherlands, France, Spain, Mexico and Colombia
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A quality-focused process development roadmap for ATMPs

Advanced Therapy Medicinal Products (ATMPs) are becoming a reality, and they have the potential to revolutionise the biotech industry.

However, of all ATMPs that have been authorized for the European market, forty percent has already been withdrawn and more have failed more than five years, which is a worrying trend. If we want to ensure sector viability, something needs to change. Cell by Design® (CBD), a cloud-based software platform that facilitates scalable, cost-effective and sustainable ATMP process development, can help.

CBD focal points
The backbone of CBD is a quality-focused process development roadmap. Risk-based process assessment to guide science and data-driven decisions, monitor the level of your technological quality objectives of the ATMP, in addition to quality, manufacturability and scalability of the developed product and process are delivered by CBD. Therefore, this is a unique step account right from the start of a development trajectory, to ensure a harmonized and robust end-to-end development process.

Finally, cost effectiveness is highly valued by CBD. By using CBD it is possible to build a design space of the process and product and to apply cheaper parameters, instead.

CBD platform
The above is captured in an intuitive cloud-based software platform that is compatible with industry standards (process monitoring, control and efficient data capturing and storage). This way of working facilitates process automation and allows for the implementation of machine learning methods and predictive modeling. The platform is in accordance with ATMP DNA documentation and is 21 CFR Part 312 compliant.

How can CBD help you?
The application of CBD can be tailored to your specific needs. It can range from a close collaboration in which you have full access to the software and can build on specialized advice in process development and regulatory challenges to any particular support you need for your specific ATMP product or process.



Setting up
CBD and Antleron share the view that there must be something better and faster than the current ATMP development system. By combining the strengths of both companies and optimizing and streamlining between production development systems and QM, regulatory affairs and validation in the system QBDs, Cell by Design has been born.

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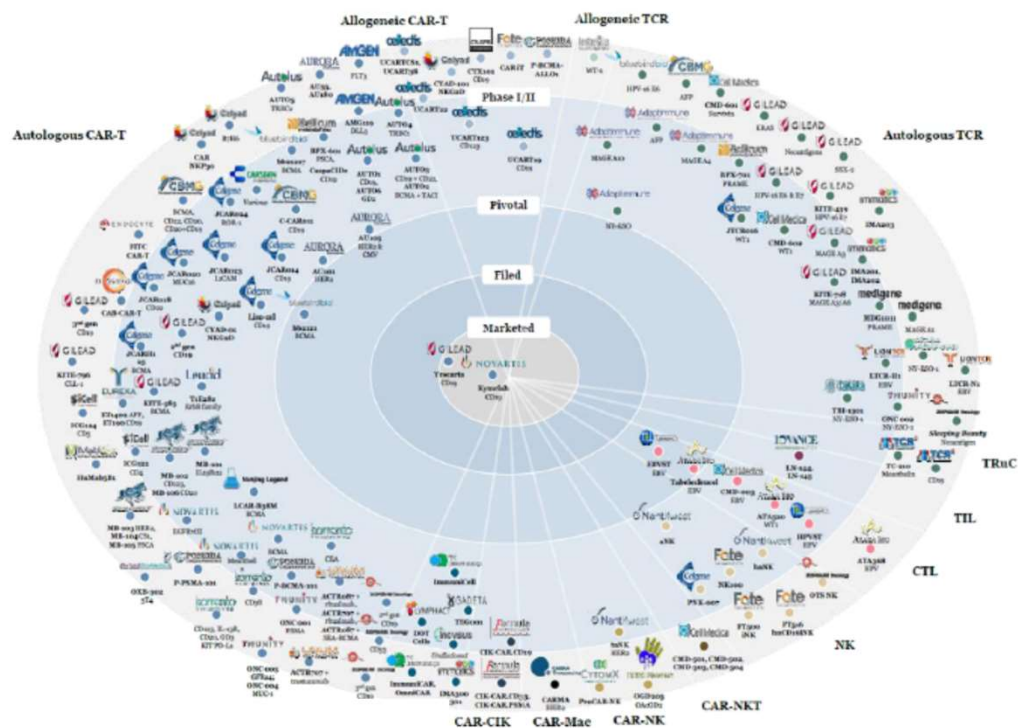
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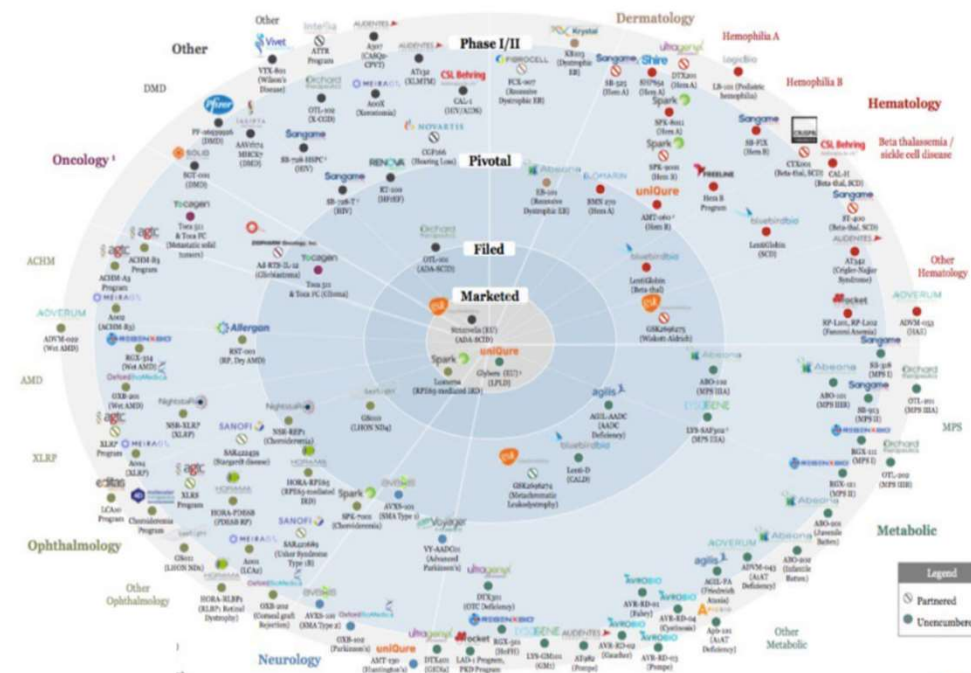


Immune-oncology Landscape



Source: Wells Fargo Securities/Cryoport

Gene Therapy Landscape

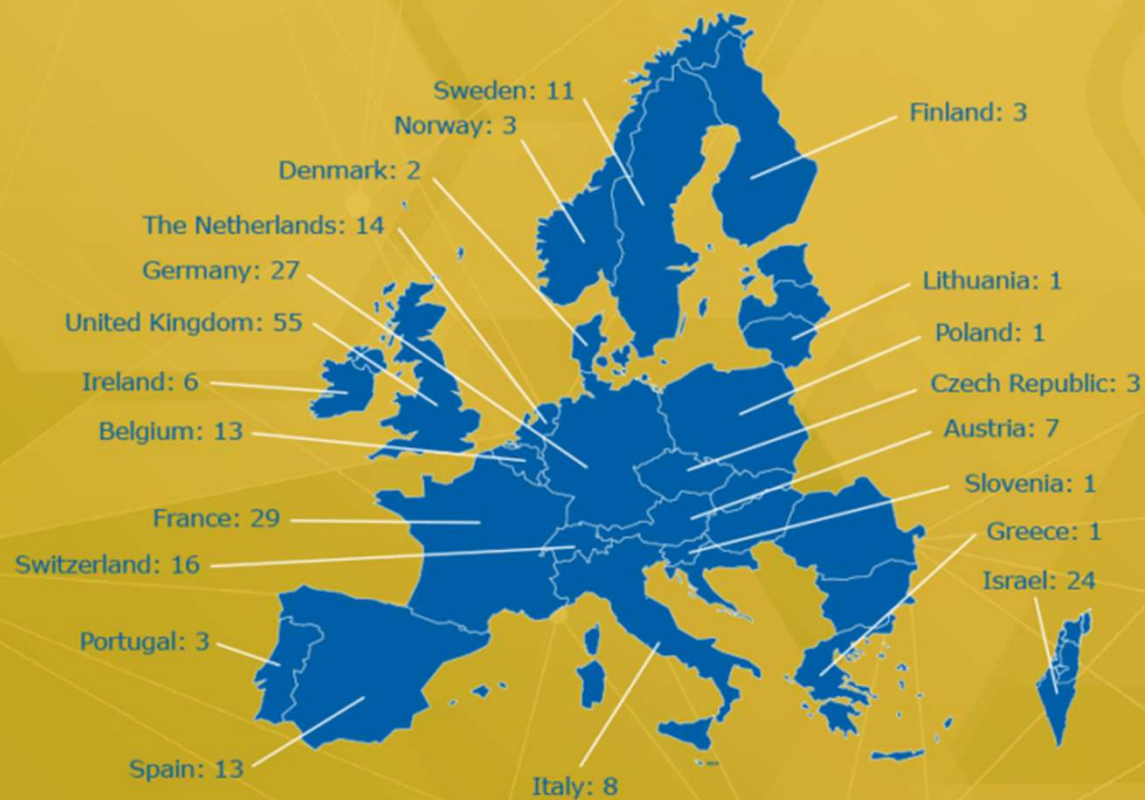


Source: Wells Fargo Securities/Cryptoport

European Sector Landscape

241+

Regenerative Medicine /Advanced Therapies Companies based in Europe/Israel





Interregional partnership for Smart Specialisation on PERSONALISED MEDICINE

Leaders

Led by **Flanders (BE)**, **Limburg (NL)** and **East-Netherlands (NL)**, the partnership engages the participation of

4 REGIONS AND MEMBER STATES

The main objective of the partnership is to realise the potential of personalised medicine by bringing solid ecosystems that combine biomedical, technological and data driven expertise. The area aims to boost to realise innovative approaches for health and care.



Reference topics



Key factors

- PM will bring the right treatment to the right person at the right time. This will not only guarantee more effective treatments, but also reduce the pressure on healthcare systems.
- PM will make preventive medicine a reality, resulting in more healthy-life years and in lowering the burden of chronic diseases.
- Cell-based personalised therapies created by converging technologies are opening new ways for treating complex chronic diseases. These approaches are also very promising for spin-out activities and job-creation.
- Building a Personal Data Management platform will contribute to citizen involvement in research and development. A platform that operates along strict safety, privacy and ethical standards as a base for trust, while addressing the needs of standardisation of data capturing, storage and exchange that open new opportunities for research and personalised services.

30 TRIALS IN TOTAL INCLUDING 19 MULTINATIONAL TRIALS

By product type:

Tissue-Engineered Product: 7 trials
Somatic Cell Therapy Product: 2 trials
Gene Therapy Product: 16 trials
Chimeric Antigen Receptor
T Cell Therapy: 5 trials

By phase:

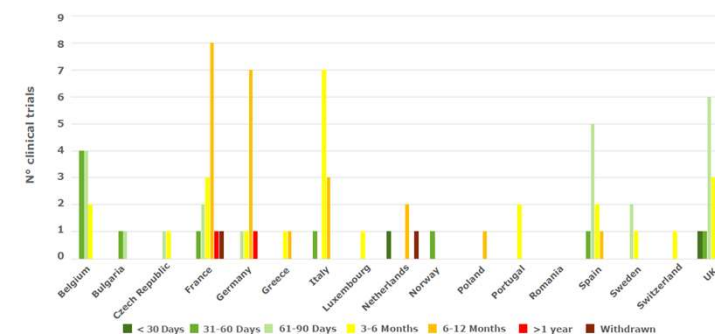
Phase I : 3
Phase I/II : 9
Phase II : 9
Phase II/III : 1
Phase III : 8



According to respondents, the most important criterion for selecting a clinical trial site and a country is the expertise and the skills of the clinical centers and healthcare professionals, followed by the speed of approval by regulatory authorities, and the quality of the review or expertise of the regulatory authorities.

Approval Times by Country — Overview

N = 26 ATMP Clinical Trials, of which 18 multinational trials



ATMP Challenges



Product Manufacturing Development

- Cost effective scale-up
- QC/Analytical assays
- Product storage



Regulatory Pathway

- Uncertain, complex regulations
- Non-conventional product development
- Product-specific considerations



Health Economics & Market Access

- Reimbursement strategies
- Pricing
- Limited comparative effectiveness data

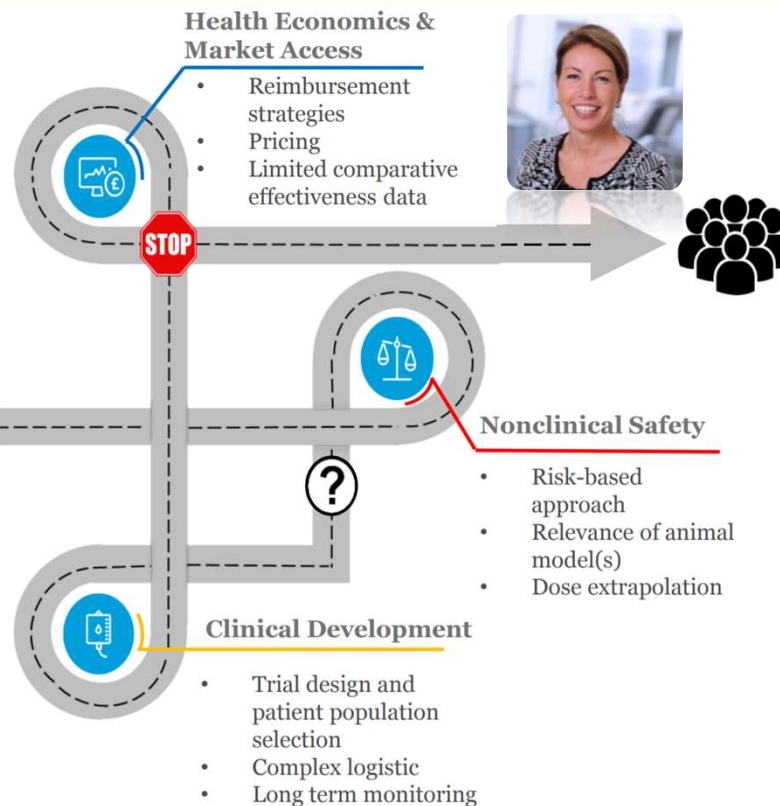


Nonclinical Safety

- Risk-based approach
- Relevance of animal model(s)
- Dose extrapolation

Clinical Development

- Trial design and patient population selection
- Complex logistic
- Long term monitoring





End to end approach





We wish you a very informative morning session!

