

# Vectorized Antibodies for In Vivo Expression by DNA and mRNA: a landscape analysis of stakeholders, technologies, targets, business and financing from an industry perspective

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## Abstracts

Vectorized Antibodies for In Vivo Expression by DNA and mRNA: a landscape analysis of stakeholders, technologies, targets, business and financing from an industry perspective

This report provides you with a landscape description and analysis of direct in vivo delivery of nucleic acid-encoded antibodies employing DNA and mRNA platform technologies from an industry perspective as of July 2021. In vivo gene-encoded antibody delivery is an elegant approach to address many of the limitations of conventional therapeutic antibodies. The three main approaches for antibody vectorization are: Adenoassociated virus (AAV) vector for delivery of DNA: AAV DNA; Synthetic plasmid DNA utilizing an electroporation device to enhance transfection efficiency after IM delivery: plasmid DNA; mRNA formulated in lipid nanoparticles (LNP): LNP mRNA.

The report brings you up-to-date with information about and analysis of

Approaches of in vivo expression of therapeutic antibodies;

Stakeholders in the field: technology and major pharmaceutical companies and investors;

Gene therapy technologies for antibody vectorization: DNA, mRNA;

Delivery technologies: adenoassociated virus (AAV) vector; electroporation; lipid nanoparticles (LNPs)

Targets and therapeutic indications selected for development of vectorized antibodies;

Preclinical and clinical experience with selected vectorized antibodies;

Financing situation of technology companies and key investors in the field

Partnering deals with financial terms;

Business strategy: indications, development path, technology partnering, investment case;

Major pharmaceutical companies: in-house technologies, R&D, collaborations.

Passive immunotherapy with conventional recombinant monoclonal antibodies has become a clinically and commercially extremely successful treatment modality. Breakthroughs in recombinant antibody technologies have resulted in the regulatory approval and commercialization of over 100 monoclonal antibodies (mAbs) to treat a variety of diseases. Sales of recombinant therapeutic antibodies in the year 2020 exceeded US\$ 184 bln (+11% vs previous year).

However, there exist a number of limitations and challenges for passive immunotherapy. Manufacturing of recombinant antibodies requires large volumes, costly production and complex protein characterization. Delivery challenges must also be overcome as in vivo administration of mAb biologics often requires high doses (grams of mAb) to achieve therapeutic efficacy, frequently at a high cost. Bioprocess manufacturing and purification can be lengthy and costly. mAbs requiring higher doses need to be administered through slow intravenous (IV) infusions to limit infusion reactions. IV delivery frequently requires hours of clinical monitoring and may involve post-infusion monitoring for allergic or anaphylactic reactions, further increasing the medical personnel required and costs of administration. Subcutaneous (SC) delivery has advantages for lower dose antibody delivery. However, SC delivery is associated with pain related to injection volume and injection site reactions, and absorption is slow due to reliance on the lymphatic system for biodistribution.

Another limitation of recombinant antibodies is their relatively short half leading to the need for repeated administration in case of chronic therapy which can be inconvenient for local administration into the eye (e.g. intravitreal). The blood-brain barrier (BBB) is a special concern for recombinant antibody therapy. The BBB prevents antibody entry to the central nervous system (CNS). Furthermore, antibodies do not enter the intracellular proteome. Systemically administered recombinant antibodies also have a potential for unspecific or toxic off-target effects.

Antibody vectorization intends to overcome such limitations of conventional passive immunotherapy. Each of the three different approaches (AAV DNA, plasmid DNA and mRNA) for in vivo expression of antibodies has its specific profile of advantages and disadvantages.

This report evaluates the industry landscape of antibody vectorization with optimized technologies for direct in vivo delivery of synthetic nucleic acid-encoded antibodies. The report is based on the identification and description of 26 companies with activities in the field of in vivo expressed therapeutic antibodies.

For each vectorized antibody technology company, a profile has been elaborated providing information about the company background/history, the financial situation, relevant technology, partnering deals and target & pipeline overview. Short profiles of the major pharmaceutical and antibody technology companies are combined in a separate paragraph. The company profiles are preceded by a chapter of stakeholder analysis.

The analysis of the three major technologies for antibody vectorization (AAV DNA, plasmid DNA and mRNA) is followed by profiles of 14 technologies in more detail (Chapter "Profiles of Vectorized Antibody Technologies").

Eventually, this report has profiled 15 product candidates for in vivo expression of antibodies in preclinical and clinical stages of R&D. The descriptions can be found in the chapter „Profiles of Vectorized Antibody Product Candidates“ in alphabetical order by the drug code or generic name, separately for each of the three main technologies.

All information in the three chapters of Company Profiles, Technology Profiles and Drug Candidate Profiles are fully referenced with 46 scientific references, in many cases with hyperlinks leading to the source of information (abstracts, Posters, papers). Non-scientific references, such as press releases, annual reports or company presentations, are disclosed within the text with an embedded hyperlink leading to the online source of

information.

Details about R&D strategy, collaboration and licensing agreements, financing rounds & sources are described in the company profiles.

What will you find in the report?

Profiles of antibody vectorization technology companies active in the field;

Description of major pharma's/biotech's role in the field (in-house R&D, partnering and investing);

Comprehensive description and analysis of emerging vectorized antibodies;

Pharmacologic profiles of selected vectorized antibodies;

Characterization, profiling and state of antibody vectorization technologies;

Target and indication selection for each antibody vectorization technology;

Description and analysis of financing rounds (capital raised, investors);

Economic terms of collaboration and licensing deals;

Sources of financing.

Who will benefit from the report?

Venture capital, private equity and investment managers;

Managers of Big Pharma venture capital firms;

Financial analysts;

Business development and licensing (BDL) specialists;

CEO, COO and managing directors;

Corporate strategy analysts and managers;

Chief Technology Officer;

R&D Portfolio, Technology and Strategy Management;

Clinical and preclinical development specialists.

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