

Gene Therapy Medicinal Products (GTMPs) represent a paradigm shift in health care as they have great potential for preventing and treating rare diseases with high unmet medical need.

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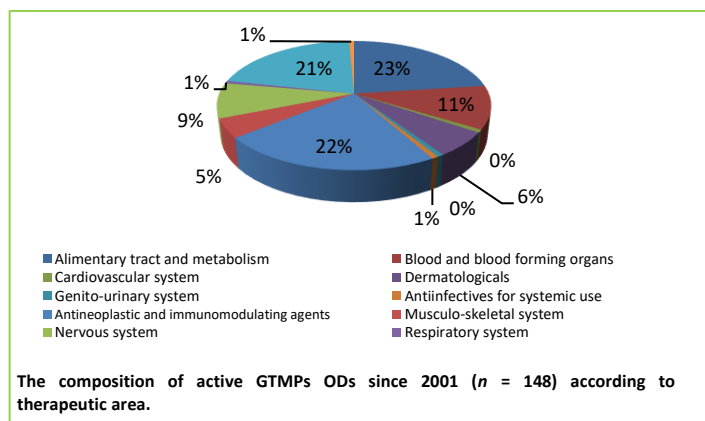
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AIM OF THE STUDY

The aim of this retrospective analysis was to provide an overview of GTMPs development in Europe. The study sample includes all medicinal products (148) that were granted a European orphan designation between 2001 and 2019 and which complied with the definition of GTMPs as defined by the Advanced Medicines Regulation. The following parameters were assessed: therapeutic area targeted, viral vector used and stage of development.

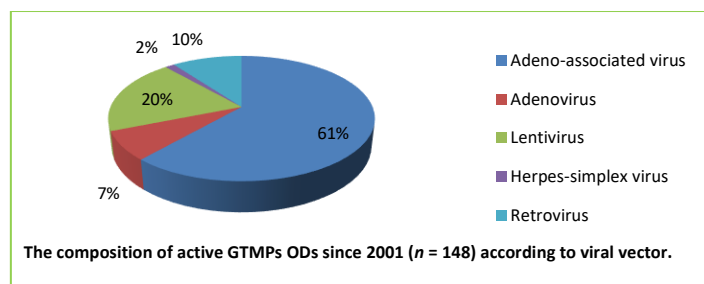
RESULTS

Of the 148 GTMPs with active orphan drug designation, treatment of metabolic disorders represents the largest group (23%), followed by antineoplastic and immunomodulating agents, treatment of defects in sensory organs and blood and blood forming organs (22, 21 and 11% respectively). All other therapeutic areas are less represented (below 10%).

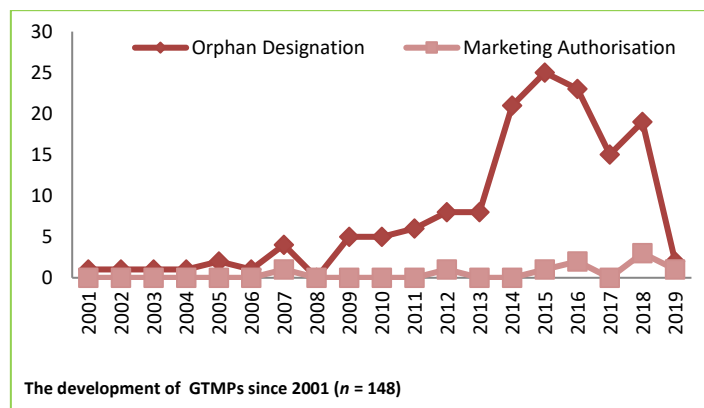


It was noted that adeno-associated viral vectors are the mostly used in gene therapy (61%),

followed by lentiviral and retroviral vectors (20% and 10% respectively).



The number of GTMPs submitted has increased over time: from 7% of OD GTMPs between 2001-2008, to 93% between 2009-2019 and coincides with the introduction of the Advanced Therapies regulation in 2008. It was however noted that only 9 GTMPs received an EU MA in the following 10yrs, which is considered low when compared to the authorization rates of other types of medicines.



CONCLUSIONS

There is a need to enhance the scientific and clinical understanding of these medicines to help improve the possibility of bringing them to licensing and to patients where no other options exist. Special attention and development could be focused on other rare diseases due to a single gene defect which have received little attention, in order

to provide patients lasting therapies and even cures for diseases that are still untreatable or for which only temporary or suboptimal treatments are available.