Trends in Gene Therapy

Description: The number of gene therapy products in preclinical to Phase III and beyond stages of development doubled between 2012 and 2015. Additionally, three gene therapy products have received regulatory approval in Europe. While these approvals give some validation to gene therapy as a therapeutic strategy, doubts remain around their return on investment.

This report addresses the following questions:

- Which therapy areas are gene therapies targeting and which programs are most advanced?
- Which delivery systems are used the most and how do the approaches differ by disease?
- What are the key features of the regulatory requirements for gene therapy products in the US and Europe?
- What differences have been observed in the approvals for gene therapy products between the US and Europe?
- What patient access obstacles have approved gene therapy products faced so far and how will current funding models have to change to facilitate access to gene therapies in the future?
- How are acquisitions and partnerships changing the gene therapy landscape?

Contents:

EXECUTIVE SUMMARY

1. The number of gene therapy products in development has doubled since 2012
2. Most products in development are in vivo therapies, except in oncology
3. The adeno-associated virus is the most popular viral vector
4. Cancer is the most common target for gene therapies in development, followed by monogenic diseases
5. Most products in advanced clinical development target cancer indications
6. Immunotherapy and oncolytic virotherapy are promising approaches in cancer
7. Regulatory and reimbursement strategies will be key to the success of new therapies
8. Gene therapy of cancer is the most active area of commercial deal-making

GENE THERAPY STRATEGIES

9. Introduction to gene therapy
10. Bibliography

GENE THERAPY PRODUCTS IN COMMERCIAL DEVELOPMENT IN 2015

11. Cancer is the most common target for products, followed by monogenic diseases

INNOVATIONS IN GENE DELIVERY TECHNOLOGIES

12. Viruses are efficient gene delivery vectors, but pose several challenges
13. Viral vectors can stimulate the host's immune system with undesirable effects
14. Plasmids as gene vectors
15. Bacteria as gene vectors
16. Cells as gene vectors
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GENE THERAPIES FOR CANCER

19. Conventional cancer treatment has limited long-term success
20. A total of 201 cancer gene therapy products are in commercial development
21. Immunotherapy is a popular broad anticancer strategy
22. Other approaches to cancer gene therapy
23. Targeted destruction of tumors encompasses a variety of approaches
24. Oncolytic virotherapy offers hope to patients with inoperable tumors
25. Anti-angiogenic gene therapies offer an alternative approach
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GENE THERAPIES FOR MONOGENIC DISEASES

27. There are 102 gene therapy products in commercial development
28. Lipoprotein lipase deficiency
29. Adenosine deaminase deficiency
30. Inherited retinal dystrophies
31. X-linked childhood cerebral adrenoleukodystrophy
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36. Cardiovascular disease
37. Sensory diseases
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39. Other diseases
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REGULATORY ISSUES

41. Introduction
42. Regulatory framework in the EU
43. Regulatory framework in the US
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REIMBURSEMENT ISSUES

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46. An alternative to a high single payment may be annuity payments for effective treatment
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49. Glybera's reimbursement struggles reveal uncertainties around long-term effects to be a key concern for payers
50. Imlygic struggles to gain reimbursement amid increased competition within melanoma
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