By the Numbers: Curative Therapies

Section Editor: The ISPOR Student Network

Milestones in Gene Therapy

1972 First time gene therapy is suggested as treatment for genetic diseases by researchers as reported in the journal 'Science.'1

1990 A four-year-old girl with severe immunodeficiency became the first patient to undergo gene therapy in the United States.2

1999 An American patient Jesse Gelsinger dies following a gene therapy experiment, causing US regulators to put some key experiments on hold, hence pushing the field back several years.3

2002 Leukemia cases are diagnosed in French children undergoing gene therapy for genetic immunodeficiency, bringing further scrutiny to the field of gene therapy.4

2003 The world's first gene therapy is approved in China for the treatment of head and neck cancer.5

2007 Doctors carry out the world's first operation using gene therapy to treat a serious sight disorder caused by a genetic defect.6

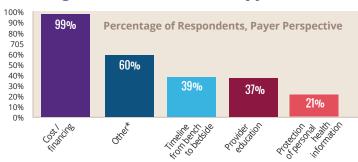
2012 Europe approves Glybera, the first gene therapy in a Western market, for an ultra-rare blood disorder.7

2016 Europe approves Strimvelis for a very rare type of immunodeficiency.8

2017 US-FDA approval brings first gene therapy to the United States - CAR T-cell therapy to treat certain children and young adults with B-cell acute lymphoblastic leukemia.9

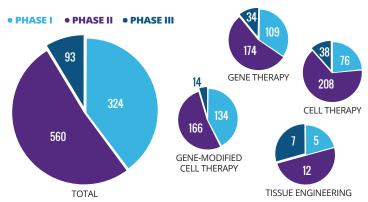
References: 1 Friedmann T, Roblin R. Gene therapy for human genetic disease? Science. 1972;175 (4025):949-55; 2Medicine's 4-year-old pioneer. The Washington Post. Available from: https://www. washingtonpost.com/archive/lifestyle/wellness/1990/09/25/medicines-4-year-old-pioneer/ 36e910ec-ff3f-405f-b5d2-c394179b4e8e/?noredirect=on&utm_term=.ce4625e20044 [Accessed February 24, 2019]; ³The Biotech Death of Jesse Gelsinger. The New York Times Magazine. Available from: https://www.nytimes.com/1999/11/28/magazine/the-biotech-death-of-jesse-gelsinger.html [Accessed February 24, 2019]; ⁴Marwick C. FDA halts gene therapy trials after leukaemia case in France. BMJ. 2003;326(7382):181.; 5Pearson, S, et al. China approves first gene therapy. Nature Biotechnology. 2004;22(1):3-4.; ⁶Blind British man in world's first operation to deliver modified DNA to his eyes. The Telegraph. Available from: https://www.telegraph.co.uk/science/2017/03/20/ blind-british-man-worlds-first-operation-deliver-modified-dna/ [Accessed February 24, 2019]; ⁷Europe approves high-price gene therapy. Fox News. Available from: https://www.foxnews.com/ health/europe-approves-high-price-gene-therapy [Accessed February 24, 2019]; 8New gene therapy for the treatment of children with ultra-rare immune disorder recommended for approval. European Medicines Agency Press Release. Available from: https://www.ema.europa.eu/en/news/ new-gene-therapy-treatment-children-ultra-rare-immune-disorder-recommended-approval. [Accessed February 24, 2019]; °FDA approval brings first gene therapy to the United States. Available from: https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm574058.htm [Accessed February 24, 2019]; 10Trends in Health Care: Disruptors and Opportunities, Summary of Findings. Available from: http://www.amcp.org/WorkArea/DownloadAsset.aspx?id=24077 [Accessed February 24, 2019]; 11Q2 2018 Data Report - Alliance for Regenerative Medicine. Available from: https://alliancerm.org/publication/q2-2018-data-report/ [Accessed February 12, 2019]

Challenges to Innovative Therapy Access¹⁰

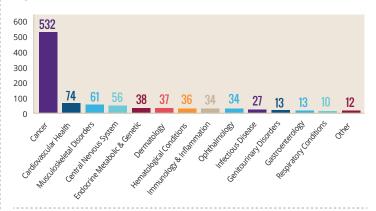


*reimbursement mechanisms, consumer expectation management, and robustness/believability of data

Number of ongoing Clinical Trials by type of Curative Therapy (as of Q2 2018)¹¹



Number of ongoing Clinical Trials (CTs) by Indication (as of O2 2018)¹¹



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