The Promise and Perils of Biotech in Personalised Healthcare. Can New Regulatory Pathways Protect the Vulnerable?

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References

Wilson, J. M. (2017). 2017 Was the Year We Have Been Waiting For. *Human Gene Therapy Clinical Development*, 28(4), 165-166.

EMA (2017). Advanced Therapies in Europe. Presentation at the 6th EBE Annual Conference on ATMPs – 5 December 2017, London.

 $\underline{https://www.ebe-biopharma.eu/wp-content/uploads/2017/12/2017-AHS-pesentation-6th-EBE-Annual-Regulatory-Conference-5-Dec-17.pdf}$

Kuehn, B. M. (2017). The promise and challenges of CAR-T gene therapy. *JAMA*, 318(22), 2167-2169.

Bach, P. B., Giralt, S. A., & Saltz, L. B. (2017). FDA Approval of Tisagenlecleucel: Promise and Complexities of a \$475 000 Cancer Drug. *Jama*, 318(19), 1861-1862.

Maschke, K.J., Gusmano, M. K., & Solomon, M. Z. (2017). Breakthrough Cancer Treatments Raise Difficult Questions, *Health Affairs* 36 (10), pp. 1698-1700.

FDA (2004). Innovation or Stagnation: Challenge and opportunity on the critical path to new medical products. http://wayback.archive-

 $\underline{it.org/7993/20180125035500/https://www.fda.gov/downloads/ScienceResearch/SpecialTopics/CriticalPathInitiative/CriticalPathOpportunitiesReports/UCM113411.pdf$

EMA (2010). Road Map to 2015: The European Medicines Agency's contribution to science, medicines and health.

http://www.ema.europa.eu/docs/en GB/document library/Report/2011/01/WC500101373.pdf New York Times Editorial Board (2018). Easier drug approval: At what price? *New York Times*, June 9.

https://www.nytimes.com/2018/06/08/opinion/drug-approval-cutting-prices.html

Kim, C., & Prasad, V. (2016). Strength of Validation for surrogate end points used in the US Food and Drug Administration's approval of oncology drugs. *Mayo Clinic Proceedings* 91(6), 713-725.

Carpenter, D., Zucker, E. J., & Avorn, J. (2008). Drug-review deadlines and safety problems. *New England Journal of Medicine*, 358(13), 1354-1361.

Honig, P. K., & Hirsch, G. (2016). Adaptive Biomedical Innovation. *Clinical Pharmacology & Therapeutics*, 100(6), 574-578.

United States Government Accountability Office (2016). FDA expedites many applications, but data for postapproval oversight need improvement. Publicly Released: Jan 14, 2016. https://www.gao.gov/products/GAO-16-192