

ASCPT RWD/RWE 25Apr2022 Webinar References:

1. Dagenais S, et al. Use of Real-World Evidence to Drive Drug Development Strategy and Inform Clinical Trial Design. *Clin Pharmacol Ther.* 2022;111:77-89. <https://doi.org/10.1002/cpt.2480>
2. 21st Century Cures Act: <https://www.congress.gov/114/bills/hr34/BILLS-114hr34enr.xml>
3. FDA Science and Research Special Topics on Real World Evidence: <https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence>
4. Framework for FDA's Real World Evidence Program: <https://www.fda.gov/media/120060/download>
5. Arondekar B, et al. Real-World Evidence in Support of Oncology Product Registration: A Systematic Review of New Drug Application and Biologics License Application Approvals from 2015–2020. *Clin Cancer Res.* 2022;28(1):27–35. <https://doi.org/10.1158/1078-0432.CCR-21-2639>
6. Baumfeld Andre E, et al. Trial designs using real-world data: The changing landscape of the regulatory approval process. *Pharmacoepidemiol Drug Saf.* 2020;29:1201– 1212. <https://doi.org/10.1002/pds.4932>
7. Purpura CA, et al. The Role of Real-World Evidence in FDA-Approved New Drug and Biologics License Applications. *Clin Pharmacol Ther.* 2022;111:135-144. <https://doi.org/10.1002/cpt.2474>
8. Broder MS, et al. Incidence and prevalence of neuroendocrine tumors of the lung: analysis of a US commercial insurance claims database. *BMC Pulm Med.* 2018;18:135. <https://doi.org/10.1186/s12890-018-0678-5>
9. Wallin MT, et al. The prevalence of MS in the United States: A population-based estimate using health claims data. *Neurology*, 2019; 92(10): e1029–e1040. <https://doi.org/10.1212/WNL.0000000000007035>
10. Chehade M, et al. Diagnostic Delay in Patients with Eosinophilic Gastritis and/or Duodenitis: A Population-Based Study. *J Allergy Clin Immunol Pract.* 2021;9(5):2050-9 e20. <https://doi.org/10.1016/j.jaip.2020.12.054>
11. Yee SW, et al. Drugs in COVID-19 Clinical Trials: Predicting Transporter-Mediated Drug-Drug Interactions Using In Vitro Assays and Real-World Data. *Clin Pharmacol Ther.* 2021;110(1):108-22. <https://doi.org/10.1002/cpt.2236>
12. Colorectal Cancer — Cancer Stat Facts: <https://seer.cancer.gov/statfacts/html/colorect.html>
13. Echle A, et al. (2021). Deep Learning for the detection of microsatellite instability from histology images in colorectal cancer: a systematic literature review. *ImmunoInformatics*, 100008. <https://doi.org/10.1016/j.immuno.2021.100008>
14. Lorenzi M, et al. Epidemiology of microsatellite instability high (MSI-H) and deficient mismatch repair (dMMR) in solid tumors: a structured literature review. *Journal of Oncology*, 2020. <https://doi.org/10.1155/2020/1807929>
15. Schomaker S, et al. Serum glutamate dehydrogenase activity enables early detection of liver injury in subjects with underlying muscle impairments. *PLoS One.* 2020;15(5):e0229753. <https://dx.doi.org/10.1371/journal.pone.0229753>
16. FDA Guidance: Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment Guidance for Industry. <https://www.fda.gov/media/92233/download>
17. Larkindale J, et al. Development of a regulatory-ready clinical trial simulation tool for Duchenne muscular dystrophy. https://c-path.org/wp-content/uploads/2021/04/Development-of-a-regulatory-ready-clinical-trial-simulation-tool-for-Duchenne-muscular-dystrophy_Poster_WMS_2020.pdf