

Case Study



Using Real-World Evidence to Accelerate Drug Approvals for Rare Diseases

First FDA Approval of a First-Line Therapy for Metastatic Merkel Cell Carcinoma



Expedience: Data collected daily from practices across the country representing physicians treating thousands of patients



Oncology-Specific EHR Depth and Breadth: Real-time tracking of broad and granular clinical and reimbursement data elements supports clinical outcomes research and development



Deep Oncology Expertise: Engagement with a robust network of physicians delivers clinical insights, enables high-level patient targeting, and the ability to hone in on rare diseases and genetic mutations

The first-line therapy indication for metastatic Merkel cell carcinoma (mMCC) was completed using real-world data and insights¹ to gain the first-ever FDA approval for this rare disease. The biopharma company sponsor needed to assess patient response to treatment for mMCC, an uncommon yet aggressive skin cancer that has one of the highest mortality rates across all skin cancer types. However, as with many rare diseases, mMCC has small patient populations and short survival times, therefore performing a traditional randomized clinical trial was not feasible. Researchers conducted a study of historical treatment therapy responses using real-world data to provide evidence of outcomes for the current standard of mMCC care and compared that to outcomes for the new therapy in a single arm trial. This study then accelerated the development of a new therapy and permitted this drug to become available to mMCC patients in need of treatment. The retrospective observational analysis enabled researchers to discover

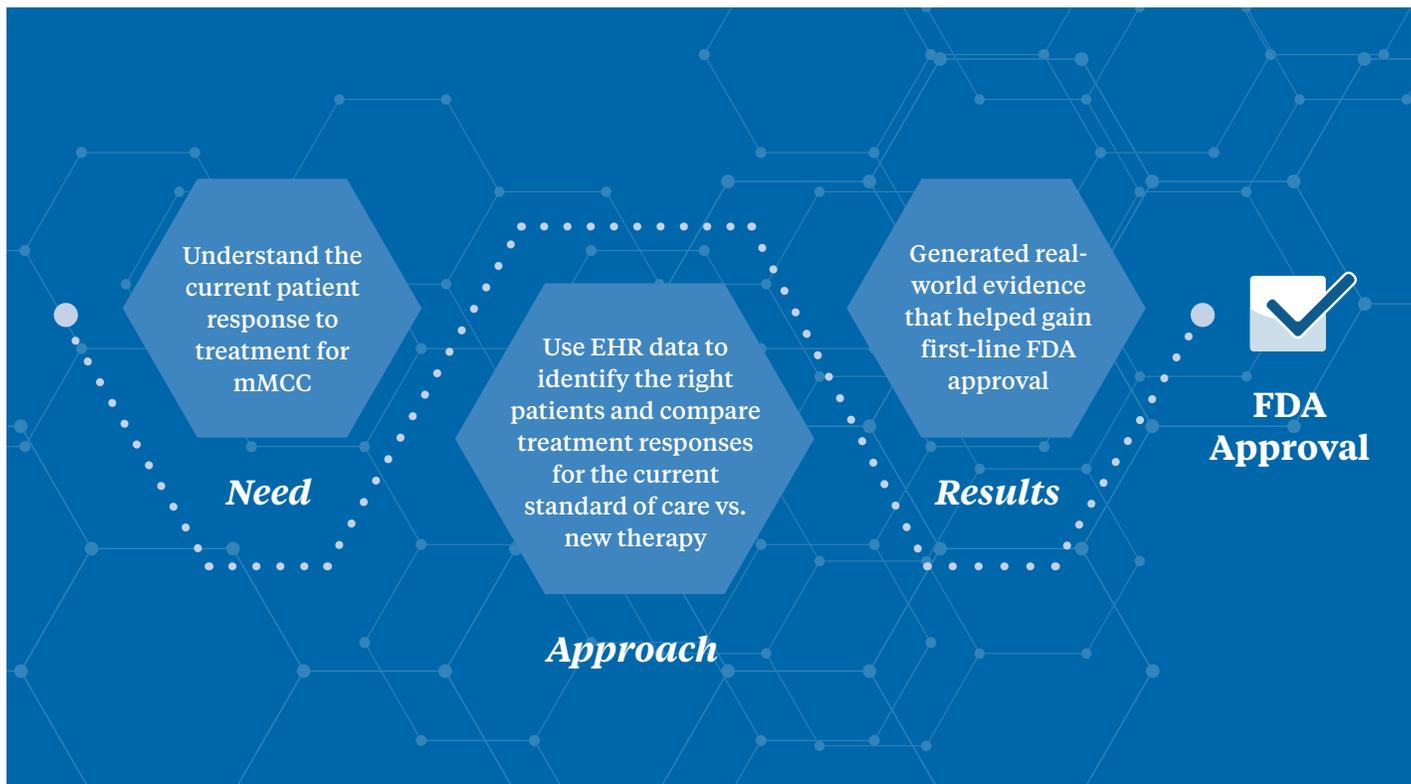
patterns in the mMCC study that may not have been visible with smaller sample sizes.

At a Glance

Need: Metastatic Merkel cell carcinoma (mMCC) has a poor survival rate, with less than half the patients surviving more than a year. Despite incidence of mMCC dramatically increasing over the last 20 years, development of an evidence-based standard therapeutic regimen was lagging. The biopharma company needed patient treatment response data to support its FDA submission for BAVENCIO® (avelumab), Avelumab a human IgG1 anti-programmed death-ligand 1 (PD-L1) monoclonal antibody. Given the short survival times for patients with mMCC, and length of time it would've taken to accrue a control arm for this rare disease, performing a traditional randomized clinical trial was not feasible. In this case, a real-world observational analysis provided the opportunity to study clinical end-points and the patient experience.

Approach: Using McKesson's EHR data, researchers identified a substantial patient population diagnosed and treated for mMCC. The iKnowMed™ EHR—used solely for oncology patients by more than 2,000

1 Cowey CL, Mahnke L, Espirito J, Helwig C, Oksen D, Bharmal M. Real-world treatment outcomes in patients with metastatic Merkel cell carcinoma treated with chemotherapy in the USA. *Future Oncol.* 2017;13(19):1699-1710.



physicians, captures outpatient medical history from community oncology practices nationwide. Through iKnowMed, 686 patients diagnosed with Merkel cell were identified, of which a third were metastatic. After applying eligibility criteria similar to those used in the clinical trial for BAVENCIO® (avelumab), in mMCC, 67 of those patients were deemed eligible for the study.² Researchers used the EHR data and medical charts to assess the response to chemotherapy in patients with distant mMCC who had received 2L+ or 1L treatment, providing real-world clinical data representing a multisite and heterogeneous sample of patients across the country.

Results: Real-world retrospective analyses¹ of patients with distant mMCC who received first-line or second-line and later chemotherapy, indicated that although responses were observed with chemotherapy, the duration of such responses was brief and associated with poor overall outcomes

in patients with mMCC. These results underscored the need for novel therapeutic approaches. With initial evidence suggesting

that immune checkpoint inhibitors have the potential to dramatically improve treatment outcomes, results were submitted and, within a year of the study's conclusion, FDA approval was granted for a new first-line therapy for mMCC.³

Differentiated Data and Expertise

Using robust EHR and economic data, McKesson delivers real-world evidence generation and observational insights that biopharma companies can use to support regulatory submissions and improved patient care. The ability to analyze robust, detailed data sets allows researchers to conduct effective pre- and post-approval studies.

By using EHR data to identify the appropriate mMCC patient population, researchers could analyze historical treatment responses and interpret the outcomes. This helped establish the first-ever FDA approval of a first-line therapy for mMCC.

1 Cowey CL, Mahnke L, Espirito J, Helwig C, Oksen D, Bharmal M. Real-world treatment outcomes in patients with metastatic Merkel cell carcinoma treated with chemotherapy in the USA. *Future Oncol.* 2017;13(19):1699-1710.

2 Kaufman HL, Russell J, Hamid O. Avelumab in patients with chemotherapy-refractory metastatic Merkel cell carcinoma: a multicenter, single-group, open-label, phase 2 trial. *Lancet Oncology.* 1-2 (2016).

3 <https://www.fda.gov/drugs/informationondrugs/approveddrugs/ucm547965.htm>

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