EVERSANA®

Patient-centric Approach to Clinical Trial Support Leveraging Medical Information Services

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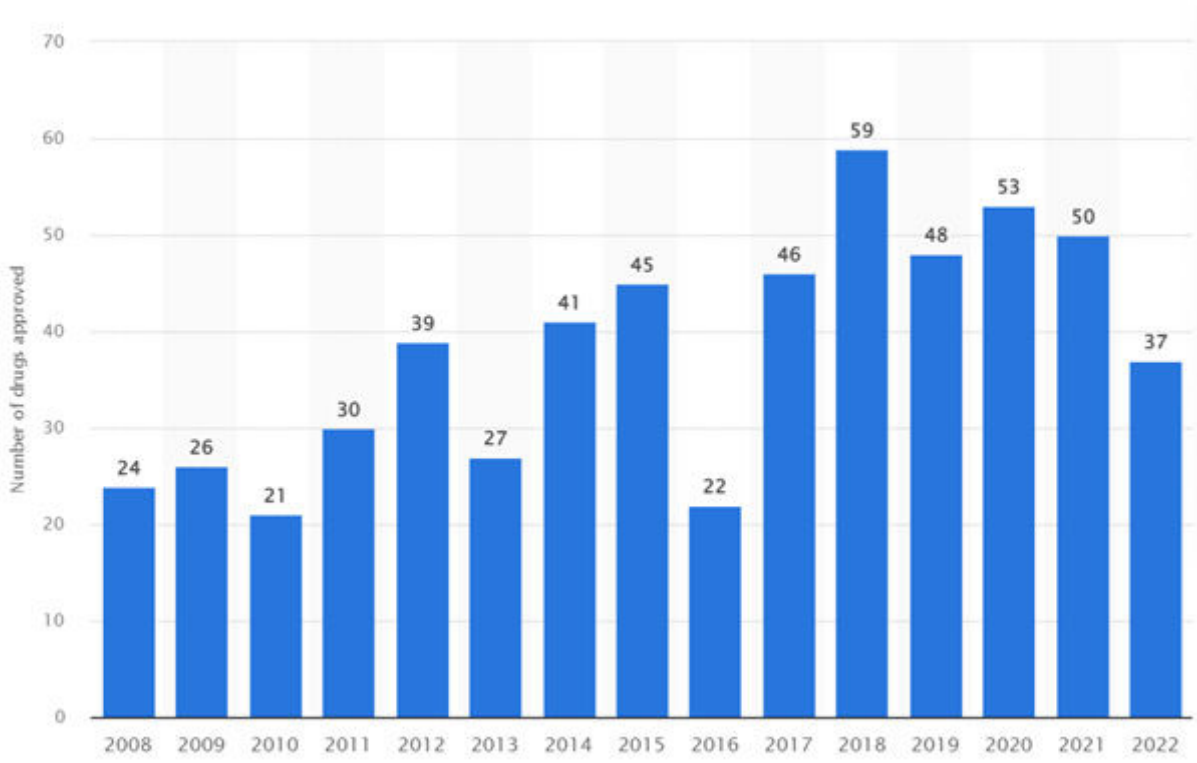
Background

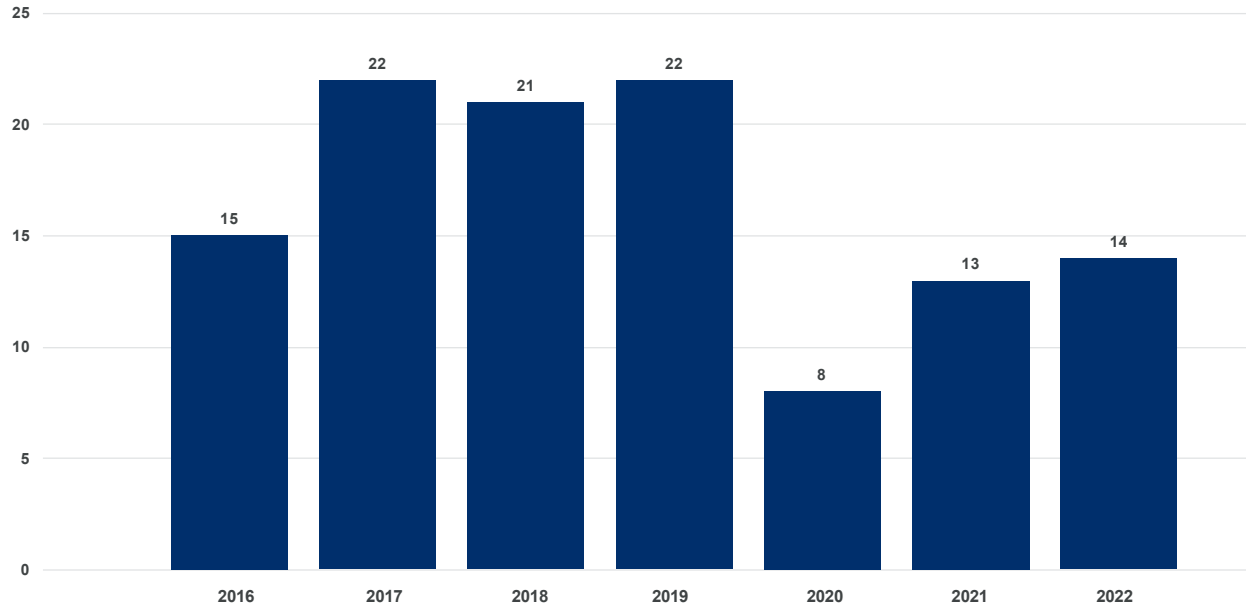
The US Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER) approves an average of 38 novel drugs annually (Figure 1). The total number of new biologics approved by the Center for Biologics Evaluation and Research (CBER) each year is on average 16 (Figure 2). Most of these new product approvals are from already established companies with existing commercialized products in the market and operations to support products in development. For example, in 2022 CDER and CBER approved 37 drugs and 14 biologics, respectively. Out of these 51 new product approvals, 16 (30%) were from emerging companies launching their first product.[1,2]

Figure 1. Total Number of Novel Drugs Approved by CDER from 2008 to 2022[1]



CDER=Center for Drug Evaluation and Research

Figure 2. Total Number of Biologics Approved by CBER from 2016 to 2022[2]



CBER=Center for Biologics Evaluation and Research

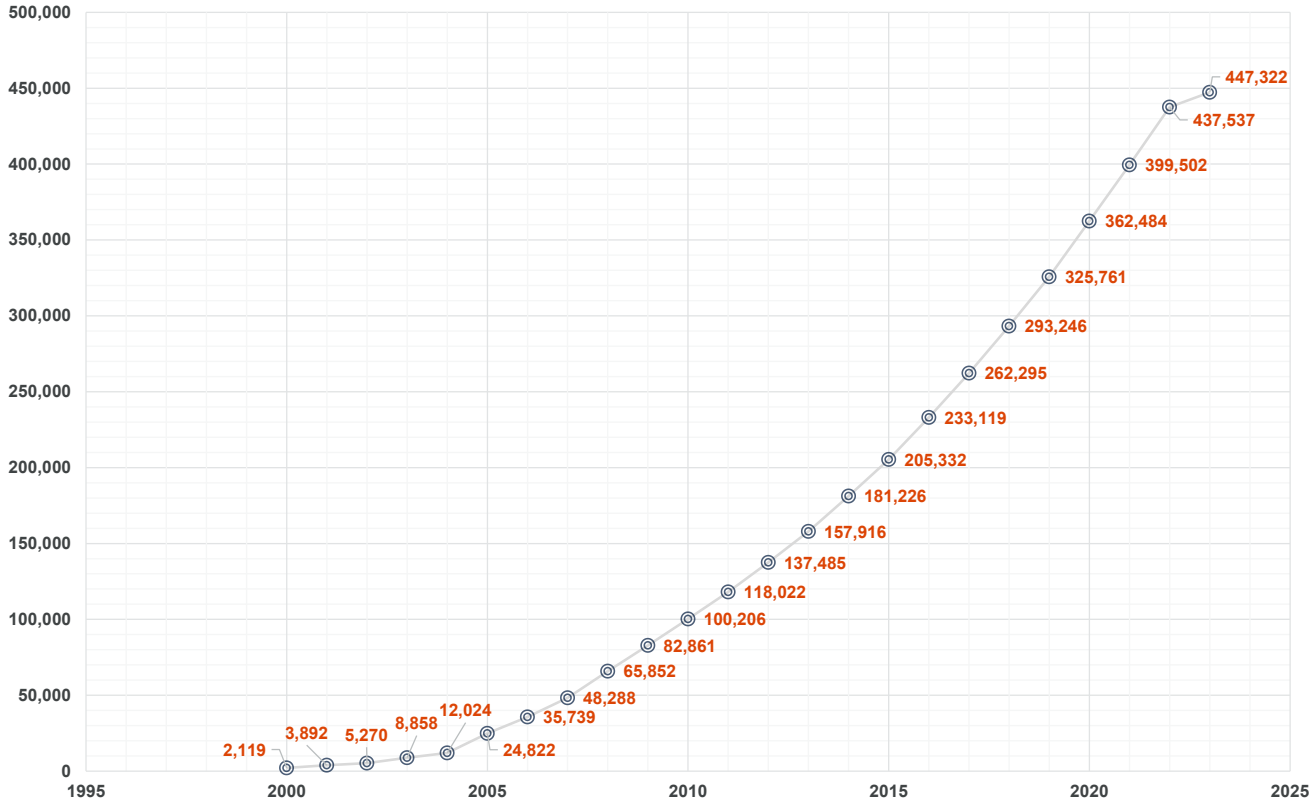
Even though the number of new drug and biologic approvals has not significantly increased year over year, the number of clinical studies conducted has increased significantly (Figure 3).[3] This trend increases competition and poses a challenge in patient recruitment, particularly for treatment targeting rare diseases, as well as cell and gene therapy, oncology, hematology and immunology. To address this challenge, it becomes imperative for companies to adopt a focused patient-centric approach well before commercialization and throughout the clinical trial recruitment process. This is especially important for emerging companies where investigators, patients and healthcare professionals may be unfamiliar with the company.

For example, in 2022, several new cell and gene therapies were approved and in 2023, additional companies are expecting favorable decisions on their regulatory submissions, including Krystal Bio, bluebird bio, Pfizer, Orchard Therapeutics, PTC Therapeutics, CARsgen Therapeutics, BioMarin, CRISPR Therapeutics, Vertex Pharmaceuticals, and GenSight Biologics. Regulatory authorities globally, including the FDA, anticipate a significant increase in the number of investigational new drug applications for cell and gene therapies each year. Some estimates suggest more than 50 gene and gene-modified cell therapies could be approved in the next few years. There are currently nearly 1,100 gene and gene-modified cell therapy trials underway.[4] The FDA is also taking steps to optimize the development of gene therapies, such as encouraging the use of biomarkers to facilitate the accelerated approval of targeted therapies for serious conditions.

Historically, the number of rare diseases has been estimated to be between 5,000 and 8,000. However, recent research conducted by RARE-X has identified as many as 10,867 rare diseases, including both genetic and non-genetic diseases. Unfortunately, many of these diseases lack clear, well-defined and standard diagnostic criteria, resulting in a significant number of patients going undiagnosed or misdiagnosed. This makes it challenging to identify eligible patients for enrollment in clinical trials, particularly when multiple companies may be vying for the same pool of patients with similar disease conditions.

The FDA has also recently issued draft guidance for the industry entitled "Clinical Trial Considerations to Support Accelerated Approval of Oncology Therapeutics". The draft guidance addresses not only the design of clinical trials but also ways to improve the data available at the time of accelerated approval. The intent is to reduce clinical uncertainty for patients by initiating post-marketing confirmatory studies promptly. However, educating patients and recruiting them in clinical trials will remain competitive and challenging.[6]

Figure 3. ClinicalTrials.gov Number of Registered Studies Over Time[3]



How EVERSANA Can Help

By adopting and optimizing a patient-centric approach, companies can improve recruitment rates and ultimately bring innovative treatments to the market more efficiently. EVERSANA's Medical Information (MI) Contact Center team provides clinical trial support services for many established and emergent biopharmaceutical companies. This patient-centric approach ensures healthcare professionals (HCPs), and patients can easily reach MI specialists with a healthcare background, who can answer questions related to the investigational product, disease state, diagnosis, and ongoing clinical trials. The MI specialists are HCPs who are highly trained in the following:

- Handling medical and scientific questions
- Identifying and handling adverse events (AEs) and product complaints (PC)
- Handling data privacy and confidentiality, including documentation of customer interactions in a 21 CFR Part 11 compliant customer relationship management database
- Communicating clinical and scientific data to patients, caregivers, patient advocacy groups, HCPs, and payers
- Providing a high level of patient-centric customer service and empathy in handling questions
- Ensuring documentation best practices and ability to generate metrics and customer insights
- Facilitating and escalating urgent clinical trial inquiries including handling of unblinding protocols

Our global MI Contact Center provides all of the following services for clinical trial support:

- Staffing to handle inquiries from contact Information on ClinicalTrials.gov to answer questions from potential study subjects and investigators
- Contact Information on study recruitment websites and advertisements

  Phone #
  Email Address
  Web Request Form
- General investigational product information and pipeline questions
- General clinical trial information, including study status
- Disease state and diagnosis information
- Clinical trial protocol information

  Inclusion Criteria
  Exclusion Criteria
- Patient recruitment

  Screening for eligibility
  Referral to study sites
  Notification of candidate referral
- Expanded access/compassionate use
- Patient outreach programs for protocol compliance
- Clinical trials emergency hotline[7]

These services also help companies conducting clinical trials in Europe meet the regulatory requirement for establishing an emergency hotline as outlined in DIRECTIVE 2001/20/EC OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL of 4 April 2001 on the approximation of the laws, regulations, and administrative provisions of the Member States relating to the implementation of good clinical practice in the conduct of clinical trials on medicinal products for human use.[7]

By setting up the MI Contact Center support during clinical development, companies can establish medical information processes and infrastructure prior to commercialization, allowing for a smooth transition and already built expertise of the MI contact center team on the product, clinical trials, and disease state.

In addition, maintaining a responsive contact center for customer communication will enhance and bolster the company's reputation with patients, healthcare professionals, and patient advocacy groups as a champion and ally in the specific therapeutic area. Furthermore, this approach establishes the processes and infrastructure for meeting post-marketing commitments for handling AEs and PCs. Most of our clients who start with our clinical trial support services transition to full medical information clients post-regulatory approval of their product. Our MI contact center is also global with multiple hubs around the world to provide both regional and global support as needed. Additionally, we offer optional after-hours coverage to provide 24/7/365 customer support.

It is crucial to optimize every stage of the patient's journey starting from clinical trial enrollment and the transition from being a study participant to a patient receiving commercial treatment. This is especially important as therapies become increasingly complex. For example, the end-to-end process behind CAR T-Cell therapy involves patient identification cell collection, storage, manufacture of the therapy, and infusion of the cell therapy back into the patient. Patients may feel overwhelmed by this process and experience anxiety about the therapy and their eligibility for a clinical trial. As more clinical trials are conducted across smaller patient populations, identifying and recruiting patients will become more competitive. Providing a positive customer experience early in the patient's journey will offer strategic advantages before and after commercialization.

Below are some case studies for clients we have provided clinical trial support for, which highlight the benefits of these services.

Case Study 1

IMAGINE THIS

In late 2018, EVERSANA was approached by a client to develop a Contact Center program to support the launch of a screening and referral service for the client's natural history trials for rare or ultra-rare inherited neurological disorders. These diseases include Charcot-Marie-Tooth Disease (CMT4J), Batten Disease (CLN5, CLN7), and Aspartylglucosaminuria (AGU). The Contact Center program included development of a medical communications management plan, workflow processes, configuration of the EVERSANA medical information system, staff training, and alignment of communication between the client and EVERSANA and was successfully established in 3 months. The program has two components; detailed screening and verification of eligible patients, and subsequent referral to trial sites. Contact center pharmacists have also been trained to answer general questions from patients, family members, or consumers regarding the disease symptoms, genetics, and research.

THE SOLUTION

The client asked EVERSANA for "high-touch" medical information services to support the complex screening and referral process for these clinical trials. The client also asked for staff highly trained and knowledgeable in the product's therapeutic area in the emerging field of gene therapy for rare neurological diseases. A mutual decision was made to use the shared pharmacist staffing model for support of this project. The shared model offers the most cost-efficient process, where the staff is highly trained to meet the client's needs while at the same time supporting other clients.

Specifically, EVERSANA pharmacists were trained to assess if the potential trial subjects had a target disease that was verified by genetic diagnosis. If this was not verified, the pharmacist directed the subject to follow up with his or her physician. Once a genetic diagnosis was ascertained, the EVERSANA pharmacist could forward the patient's information to the study site for further eligibility screening. Another challenge of this project was that subjects could be contacting EVERSANA from outside the United States. Accordingly, interpretation and translation services were made available for inquiries from non-English speaking customers.

RESULTING IN

- The client continued to work with EVERSANA as more clinical trial sites opened and as additional studies were conducted.
- The client also expressed their appreciation for our forward-thinking and flexibility in services and operations to meet their tailored, ever-changing needs.

Case Study 2

- The EVERSANA contact center has been providing clinical trial screening and referral services for a client with CAR-T therapy since 2017.
- The client requested "high touch" clinical trial / medical information contact services supported by US based pharmacists to provide a high level of knowledge while ensuring empathy when speaking with patients and family members.
- The client required a high level of sensitivity to ensuring quick escalation of any sensitive or critical issues.
- The goal was to provide a highly "patient-centric" approach to clinical trial recruitment.

- EVERSANA implemented a shared pharmacist staff model to conduct trial screening and referrals to clinical trial sites within the United States and internationally. The highly trained and compassionate team has helped patients, family members and healthcare professionals navigate through the complex clinical trial participation process and promptly facilitated successful referrals to trial sites.
- EVERSANA worked in collaboration with the client to develop resources to streamline the screening and referral process to be patient-minded and provide a high-level customer experience.
- Being involved in CAR-T clinical trials EVERSANA has been key in accessing and escalating time sensitive issues to the client.
- The client has been leveraging EVERSANA's expertise to launch their first commercial product. Since the contact center was already established, EVERSANA had the ability to quickly transition to a full-service contact center. In addition to start up and maintenance of telephony, medical information, adverse event and product complaint services, VEEVA Vault and VEEVA CRM integrations have been implemented.

- As of March 2023, EVERSANA has screened and referred over 800 patients to the clinical trial sites.
- Non-escalated clinical trial inquiries and requests have been handled within one business day.
- The client continues to work with EVERSANA on increasing support for additional clinical trials.
- Based on EVERSANA's high level of clinical trial support services, the client is planning to utilize EVERSANA for contact center services in Europe.

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