STUDY POPULATION: Conduct literature review on 1. background of HD, 2. what symptoms and outcome measures are most important to patients, including the Patient-Focused Drug Development (PFDD) meeting for HD–led by the U.S. Food and Drug Administration (FDA), 3. what outcome measure tools currently exist and what they measure. Utilizing Clinicaltrials.gov, trials for HD were examined to assess the number of trials conducted, what COAs were used, and funding types. Trials were filtered by study type (keep Interventional) and status (filter out suspended, terminated, unknown, and withdrawn). The frequency of COAs will then be mapped based on the symptoms from the PFDD meeting. RESULTS/ANTICIPATED RESULTS: From the PFDD meeting for HD, symptoms that were important to patients include cognitive impairment, depression and anxiety, and motor symptoms. From the 139 interventional studies that were active, complete, recruiting, or not recruiting, 79 studies were conducted by Industry, 3 by NIH, 93 by Other (Academia/Community Organizations), and 1 by a U.S. Federal Agency (other than NIH). One of the most commonly used COA is the Unified Huntington's Disease Rating Scale (UHDRS), which includes a motor, cognitive, and behavior assessment, and an assessment on functional capacity and independence. Of the 27 out of 139 trials analyzed to date, there were a total of 37 COAs. DISCUSSION/SIGNIFICANCE: The widespread use of UHDRS can be attributed to its standardization in 1999. It captures the symptoms of HD that are most important to patients. Because UHDRS is not sensitive to any one symptom, other COAs have been developed which focus on unique aspects of HD and allow for its earlier detection.

512

Understanding Expanded Access: Who are the Patients?

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OBJECTIVES/GOALS: The FDA allows physicians to request clinical use of investigational drugs, biologics, and devices for patients with no satisfactory treatment options through a pathway called Expanded Access (EA). TEAMSS (Transforming Expanded Access to Maximize Support and Study) sought to examine single-patient cases to better characterize these patients. METHODS/STUDY POPULATION: We prospectively collected data on requests for single-patient EA at any one of the four collaborating TEAMSS institutions (Duke University, University of Rochester, University of Michigan, and University of Texas Southwestern) between September 1, 2021 and February 28, 2023. Regulatory and health records were reviewed for past cases that occurred between June 1, 2018 and August 31, 2021. Descriptive statistics were performed on data from the submission process, the patient demographics, the indication for treatment, and patient health status over time. RESULTS/ANTICIPATED RESULTS: The patient population was representative with respect to the largest racial groups (69.3% White / 13.0% Black or African American) and legal sex (51.3% male / 48.7% female). All ages were represented, with overrepresentation of those 60-70 years old (16.8%) and under 10 (14.8%). Patients were most often treated for infectious diseases (44.2%) or oncologic conditions (39.0%). Those who received more than one dose stayed on treatment for 76 days (median) and up to 1427 days (maximum). At the end of study, 53.9% had completed treatment as planned, moved to commercial product, or continued treatment. Death, disease progression, or failure to respond occurred for 31.9% of patients. DISCUSSION/SIGNIFICANCE: The population that receives Expanded access treatments is heterogeneous in both demographics and medical conditions. Some successful treatments are continued for years. Many patients complete their treatment, and a minority experience death or disease progression during treatment.

Research Management, Operations, and Administration

514

Rapid Activation Trial (RAT) Program for High Priority Clinical Trials

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OBJECTIVES/GOALS: Mayo Clinic (MC) launched the Rapid Activation Trial (RAT) pilot program in 2022 to expedite the activation of high priority and high impact clinical trials. The objective was to develop a process for rapid activation through robust screening, prioritization, and project management (PM) support. METHODS/STUDY POPULATION: The project team developed a robust screening and approval process for the RAT program using a combination of an objective scoring tool (based on strategic priorities) and a diverse selection committee to screen and approve eligible trials. Sponsors had to commit to RAT program timelines. Upon approval, trials were prioritized at the highest level within each business unit involved in the activation process. The number of trials approved annually were limited to 8 to manage volume and facilitate seamless prioritization with an activation timeline goal of 6 weeks. Project management support for RAT program focused on financial, regulatory, logistical, and operational elements to open trials expeditiously. RESULTS/ANTICIPATED RESULTS: In 2022, thirteen (13) applications were received and eight (8) were approved by the RAT selection committee. The approved trials activated with a median open to enrollment time of 6.4 weeks from engaging with business units. They also aligned closely with organization's strategic priorities, including but not limited to Investigator Initiated Trials, Multi-Site protocols, IND/IDE protocols, Rare Diseases, First in Human and Commercialization potential trials. PI and study team feedback was positive. In 2023, the RAT program was renewed due to the pilot's significant success in 2022. The goal is to open 10 trials and 5 have been activated by the end of Q3, 2023 with a median timeline of 6 weeks. DISCUSSION/SIGNIFICANCE: Rapid activation of high priority and high impact clinical trials enables an organization to strategically prioritize and open complex clinical trials. This allows the delivery of innovative, timely cures to patients in an expeditious timeline.

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