the full academic medical center process. METHODS/STUDY POPULATION: Through the Transforming Expanded Access to Maximize Support and Study grant, we reviewed regulatory records for single-patient EA requests at four institutions (Duke University, University of Rochester, University of Michigan, and University of Texas Southwestern) which occurred between June 1, 2021 and February 28, 2023. Key data was collected, including the investigational product requested, submission and approval dates, urgency of request, and indication for treatment. Descriptive statistics were performed with Microsoft Excel. RESULTS/ANTICIPATED RESULTS: A total of 405 EA requests were identified, of which 319 (78.8%) were for drugs, 59 (14.6%) for biologics, and 27 (6.7%) for medical devices. The majority were characterized as non-emergency (60.7%), but the proportion of emergency to non-emergency cases varied considerably when stratified by year, with a peak in emergency cases in 2020. The most common products included therapies for COVID-19 and Mpox. Median time to obtain all approvals for treatment was 7 days for emergency cases and 28 days for non-emergency. The FDA review took the least time, with a median of 1 day in non-emergency cases. Full board approval from an institutional review board in non-emergency cases was 7 days. DISCUSSION/SIGNIFICANCE: These results generally align with previous reports on EA submissions received by the FDA. The timelines for the EA process represent an important benchmark both for treatment planning and institutional improvement.

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Regulatory Lens of a QA/QC Project Manager

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OBJECTIVES/GOALS: The primary purpose of the QA/QC Project Manager (PM), appointed under the NCATS UL1 administrative supplement award, is to facilitate quality and timely NCATS prior approval submissions preventing study start delays. Other goals include supporting these projects' IRB applications and monitoring to ensure data quality and compliance. METHODS/STUDY POPULATION: At the Indiana CTSI, the QA/QC PM is assigned to the Regulatory Knowledge and Support program (RKS) and functions as a unique regulatory service provider. Through monitoring, auditing, and personalized consultations, the IN CTSI QA/QC PM provides study teams with regulatory, GCP, and other compliant study conduct insights while managing NCATS prior approval and RPPR submission quality and timeliness. In contrast to many CTSAs, this role is uniquely situated within RKS and provides QA/QC support through a regulatory lens. The Indiana CTSI QA/QC PM serves on the CTSA QA/QC Lead Team collaborating with NCATS and other CTSA QA/QC personnel. The Lead Team engages with NCATS to host monthly/quarterly meetings and participate in a discussion forum of NCATS and other CTSA QA/QC personnel. RESULTS/ANTICIPATED RESULTS: Not all CTSAs employ the QA/QC PM as regulatory support and the role and skill sets at each CTSA vary, yet the collaborative nature of these individuals across the CTSAs facilitates sharing of resources and knowledge. While prior approval and RPPR submissions vary widely, the QA/QC PMs can rely on their counterparts for guidance complying with the same regulations and policies within unique research settings and institutional nuances. The IN CTSI QA/QC PM, in collaboration with the QA/QC Lead Team, provided quality assurance revisions to the NCATS prior approval instructions which were adopted and published by NCATS January 2022 for implementation at all CTSAs. Ongoing, quality control efforts are accomplished through education, monitoring, and regulatory consultations. DISCUSSION/SIGNIFICANCE: As the research environment evolves, the QA/QC PM responsibilities shift in response to needs within RKS and NCATS. The versatility of the position enables QA/QC to occur at all stages of a study. QA/QC strategies aim to facilitate communication, quality NCATS prior approval and RPPR submissions, and compliance with proposed study conduct.

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Addressing the Regulatory Needs and Challenges of Academic Researchers by Creating a One-Stop Shop Web Portal

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OBJECTIVES/GOALS: To identify challenges faced by academic researchers in accessing online regulatory information and/or tools to advance their research work to develop a free, publicly accessible, interactive web portal that provides regulatory support. METHODS/STUDY POPULATION: The Regulatory Knowledge and Support core of the Southern California Clinical and Translational Science Institute interviewed five local research professionals. These interviews guided the development of a Qualtrics survey, consisting of multiple responses and open-ended questions, submitted to our local institutional review board (IRB). After receiving IRB approval, the survey was disseminated via email, newsletters, flyers, and presentations targeting researchers at academic institutions and members of clinical and translational science hubs. Survey data will be used to identify the challenges academic researchers face in finding regulatory resources and to compile the types of regulatory information or tools they would find helpful for their research. RESULTS/ANTICIPATED RESULTS: According to the interviews, researchers with extensive involvement in clinical trials found regulatory resources easily accessible compared to those with less experience. Additionally, they all stated having a colleague or regulatory specialist whom they can consult about regulatory requirements. Insights from these initial interviews confirmed the need to obtain a comprehensive view across research professionals. Anticipated results will show the challenges in accessibility, source, and type of regulatory resources researchers typically encounter. It is also anticipated that researchers will share what kinds of resources they would find most useful for their work. Ultimately, the information and tools identified as essential by survey takers will be collected and incorporated into the web portal. DISCUSSION/SIGNIFICANCE: Academic researchers find navigating through regulatory hurdles persistently challenging when translating their work from bench to clinic, especially since academia is typically resource-constrained. Findings from this study will allow the creation of a web portal for researchers that is broadly accessible and meets their regulatory needs.

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Analysis of Clinical Outcome Assessments in Clinical Trials for Huntington's Disease

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OBJECTIVES/GOALS: Examine the use of Patient-Reported Outcomes (PRO) in Huntington's Disease (HD) clinical trials (CT) and compare across time and sponsor types. METHODS/

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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.

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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.

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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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