

NORD Summit 2020 Top 3 Poster Research Summary

Poster: Pharmacist-identified gaps in the expanded access process at an academic medical center

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Expanded access is a pathway through the Food and Drug Administration that allows the use of an investigational agent outside of enrollment in a clinical trial. Eligibility for this pathway includes having an immediate or life-threatening disease with no comparable alternatives and no feasible enrollment in a clinical trial. The barriers to enrollment in clinical trials may be geographic, eligibility criteria, or a disease state with no trials available.

Although patients receiving investigational drugs through this pathway are not research participants by definition, the lack of comparable therapeutic alternatives combined with administration of investigational agents (but without the protections offered to those enrolled in a clinical trial) designates these patients as a vulnerable population.

Additionally, safe and effective use of an investigational agent requires expertise in both pharmacotherapy and research regulations. For these reasons, we sought to characterize the use and evaluate the documentation of expanded access therapies at our academic medical center and determine potential future direction to remedy any identified gaps in the expanded access clinical monitoring process.

Of 76 single-patient investigational new drug (IND) requests that were made between 2016 and 2019 at our center, 61 met our inclusion criteria of request for an investigational drug with at least one dose administered. Overall request and patient demographics results speak to the diversity of patients receiving therapies through the expanded access pathway. The median age was 28.5 years (IQR, 10 – 57), with nearly a third (18/61) of requests for pediatric patients. Just over half of requests (34/61) were for hematology or oncology diagnoses, and approximately half of those were for targeted therapies. Eighteen percent of requests were for infectious diseases, and 7% for infectious diseases in hematology patients. Of note, 83% of completed requests were intended for use in the ambulatory setting, and 73% of completed requests were for oral administration route.

A complete clinical monitoring plan was documented for 16% of patients.

Adherence was assessed for just one quarter of patients who were eligible for an assessment. Only seven of 61 patients had a documented drug-drug interaction assessment at therapy initiation, though retrospective review of patient medication profiles revealed 70 potential drug-drug interactions in 21 of these patients. Over half of symptomatology was attributed as adverse effects of expanded access agent, further signaling a potential gap in the pharmaceutical care of these patients. Involvement of a pharmacist with dedicated role related to drug therapy obtained through expanded access pathway is noted as a future direction, as pharmacists with experience managing investigational drugs are well-suited to close the identified gaps and have the pharmacotherapy knowledge to optimize patient documentation and clinical outcomes.