Survey: Study of Pediatric Hematologists'/Oncologists' Perspectives on and Experiences with Compassionate Use

Start of Block: Block 1

T1 **Study of Pediatric Hematologists'/Oncologists' Perspectives on and Experiences with Compassionate Use** We hope you will consider participating in this study by taking this survey. The purpose is to better understand pediatric hematologists'/oncologists' perspectives on and experiences with single patient pre-approval access to investigational drugs and biologics outside of clinical trial settings (otherwise known as compassionate use).

This survey is being conducted by researchers at the Division of Medical Ethics in the Department of Population Health at NYU Langone Health, in collaboration with St. Baldrick's Foundation. Dr. Alison Bateman-House is the principal investigator. Results from the survey may help improve processes relating to the use of investigational drugs outside of clinical trials.

T2 Participating in this study is completely voluntary. The survey will take about 10-15 minutes to complete. The survey will ask about your perspectives and opinions about single pateint pre-approval non-trial access in a multiple-choice answer format. Your responses will be kept confidential and will not be linked to you, so while there is a risk to privacy, it is minimal. Only aggregate results will be reported. If you participate in the study, you may benefit by learning more about single patient Expanded Access (EA) and Right To Try (RTT) requests for investigational drugs. Submission (pressing the clearly marked forward arrow at the end of the survey) confirms your consent to participate in the study, but you can decide at any time prior to submitting your answers to not complete the survey or to not submit your completed survey.

We ask you to answer as many questions as possible, but you may skip questions that you do not want to answer. Please base your answers on your own experiences. We are looking for your opinion/experience. There are no wrong answers. After you submit your responses, you will be directed to a second survey where you have the option of entering a lottery to win one of five $100 gift cards. You may also indicate your willingness to be contacted about a future, follow-up qualitative study about this topic or about St. Baldrick's advocacy work.

If you choose to provide your name and contact information (which is optional), they will not be linked to your survey responses.

To submit your responses (which indicates your consent to participate), please press the forward arrow on last page of survey! Thank you.

Page Break

T3 This study has been approved by the NYU School of Medicine IRB. If you should have any questions or concerns about your rights as a research participant, please contact the NYU School of Medicine IRB office at 212-263-4110.

If you have any questions about this research study, please contact Dr. Alison Bateman-House, Assistant Professor, at alison.bateman-house@nyulangone.org or 646-501-2636.

End of Block: Block 1

Start of Block: Block 2

T4 **Section I. Background information**

There are currently two pathways by which single patients, with support from their physicians, can access investigational drugs and biologics for therapeutic use (outside of a clinical trial):

The FDA's Expanded Access (EA) program The federal Right To Try (RTT) pathway

Pre-approval access outside of clinical trial settings is also known as compassionate use. The primary purpose is to diagnose, monitor, or treat a patient's disease or condition, not to collect research data.

T5 Scope and limits of this survey: We are asking about your experiences with and your thoughts about the FDA's Expanded Access (EA) program and the federal Right To Try (RTT) pathway for single patient pre-approval access to investigational drugs, including biologics.

We are NOT asking about medical devices, as there are different rules for devices.

We are NOT asking about the FDA's Expanded Access programs for intermediate or widespread use, intended to provide an experimental drug or biologic to a group of patients for treatment use. We are NOT asking about "OFF LABEL" uses of approved drugs and biologics.

End of Block: Block 2

Start of Block: Block 3

T6 **Section II. Description of you and your practice.**

Q1 Are you board certified in pediatric hematology/oncology?

- ○ Yes (1)
- ○ No (2)

Display This Question:

If Are you board certified in pediatric hematology/oncology? = Yes

Q2 If you are board certified in pediatric hematology/oncology, how many years have you been board certified?

_____

Display This Question:

If Are you board certified in pediatric hematology/oncology? = No

Q3 If you are not board certified in pediatric hematology/oncology, which best describes you?

- ○ I am currently a resident; planning to pursue career in pediatric hematology/oncology (1)
- ○ I am currently a fellow in pediatric hematology/oncology (not yet board eligible) (2)
- ○ I am a pediatric hematologist/oncologist that is board eligible (6)
- ○ I am a medical doctor but not a pediatric hematologist/oncologist (3)
- ○ I am not a medical doctor (4)
- ○ Other; please describe (5) _____

Skip To: End of Block If If you are not board certified in pediatric hematology/oncology, which best describes you? = I am not a medical doctor

Skip To: End of Block If If you are not board certified in pediatric hematology/oncology, which best describes you? = I am a medical doctor but not a pediatric hematologist/oncologist

Page Break

Q4 What percentage of your professional time do you spend providing direct clinical care or indirect care (related administrative work) for patients? Please provide your best estimate.

- ○ 0% (1)
- ○ 1-25% (2)
- ○ 25-50% (3)
- ○ 51-75% (4)
- ○ 76-100% (5)

Skip To: End of Block If What percentage of your professional time do you spend providing direct clinical care or indirect... = 0%

Page Break

Q5 Is your clinical practice affiliated with an academic medical center?

- ○ Yes (1)
- ○ No (2)

Q6
Please describe the medical center that is affiliated with your clinical practice (check all that apply):

- ☐ Focused on care of children only (1)
- ☐ Focused on care of adults and children (2)
- ☐ Focused on cancer care (3)
- ☐ Provides care in many therapeutic areas (4)
- ☐ My clinical practice is not affiliated with a medical center (5)

Q7 Approximately how many new hematology/oncology patients are seen per year in your clinical practice?

- ○ 1-50 (1)
- ○ 51-100 (2)
- ○ 101-250 (3)
- ○ 250-500 (4)
- ○ 501-1000 (5)
- ○ over 1000 (6)

Page Break

Q8 Approximately how many pediatric oncology patients have you treated in the preceding 12 months? (Please include patients who you supported, even if you were not their primary physician.)

_____

Q9 Approximately how many of the pediatric oncology patients you have treated in the preceding 12 months have died? (Please include patients who you supported, even if you were not their primary physician.)

- ○ None (1)
- ○ 1-5 (2)
- ○ 6-10 (3)
- ○ 11-20 (4)
- ○ Over 20 (5)
- ○ Not applicable; I do not see oncology patients (6)

Page Break

Q10 Approximately how many pediatric hematology patients have you treated in the preceding 12 months? (Please include patients who you supported, even if you were not their primary physician.)

_____

Q11 Approximately how many of the pediatric hematology patients you have treated in the preceding 12 months have died? (Please include patients who you supported, even if you were not their primary physician.)

- ○ None (1)
- ○ 1-5 (2)
- ○ 6-10 (3)
- ○ 11-20 (4)
- ○ Over 20 (5)
- ○ Not applicable; I do not see hematology patients (6)

End of Block: Block 3

Start of Block: Block 4

T7 **Section III. Your perspectives on single patient access to investigational drugs, including biologics, outside of clinical trial settings**

Q12 How often do the following scenarios occur?

	Very often (1)	Often (2)	Sometimes (3)	Rarely (4)
Patient exhausts all approved therapies (8)	○	○	○	○
No approved therapies available to treat the patient (9)	○	○	○	○
Patient unable to participate in clinical trial for desired investigational drug because he/she does not meet inclusion/exclusion criteria (1)	○	○	○	○
Patient unable to participate in clinical trial for desired investigational drug because of logistical barriers (4)	○	○	○	○
Patient unable to participate in clinical trial for desired investigational drug because of financial barriers (5)	○	○	○	○
Patient unable to participate in clinical trial for desired investigational drug because there were no spots and/or the trial was no longer enrolling (6)	○	○	○	○

Patient not interested in participating in clinical trial(s) (7)	○	○	○	○

Page Break

Q13 To qualify for the FDA's single patient Expanded Access (EA) program, certain conditions must be met: Patients must have a serious or immediately life-threatening disease or condition for which there are no alternate options (which include marketed therapy or participation in a clinical trial). The potential benefit must justify the potential risks of use, and risks must not be unreasonable in the context of the disease/condition to be treated.

The FDA must ensure that providing the investigational drug will not interfere with clinical investigations that could support marketing authorization. The FDA's pathway for single patient EA requires permission from the FDA to proceed with such requests. Single patient EA requests must also be reviewed by an IRB, unless they meet specific emergency criteria.

Manufacturers/sponsors must be willing to supply the investigational drug.

Q14 *Please indicate how much you agree with the following statements regarding seeking access to investigational drugs for therapeutic use outside of clinical trials through the FDA's single patient expanded access (EA) program.*

	Strongly Agree (1)	Agree (2)	Disagree (4)	Strongly disagree (5)
If my patient meets eligibility requirements, I would consider applying, on his/her behalf, for access to an investigational drug through the FDA's EA program. (2)	○	○	○	○
I am familiar with the process by which to seek an investigational drug for my patient, through the FDA's EA program. (3)	○	○	○	○
My clinical practice/ colleagues would be supportive of my choice to seek access to an investigational drug or biologic on behalf of a patient through the FDA's EA pathway. (6)	○	○	○	○

Page Break

T8 To use the federal right to try pathway (RTT) pathway (passed into law on May 30, 2018), certain conditions must be met: Patients must have a life-threatening disease or condition Diseases or conditions where the likelihood of death is high unless the course of disease is interrupted Diseases or conditions with potentially fatal outcomes, where the end point of clinical trial analysis is survival. Patients must have exhausted approved treatment options and be unable to participate in a clinical trial involving the eligible investigational drug, as certified by a physician. To be eligible for the RTT pathway, drugs must have completed a Phase I clinical trial, and be in active clinical development and not the subject of a clinical hold. FDA approval is not required. IRB approval is not required either, but patients must provide the treating physician with written informed consent.Manufacturers/sponsors must be willing to supply the investigational drug.

Q15 *Please indicate how much you agree with the following statements regarding seeking access to investigational drugs for therapeutic use outside of clinical trials through the federal right to try (RTT) pathway.*

	Strongly Agree (1)	Agree (2)	Disagree (4)	Strongly disagree (5)
If my patient meets eligibility requirements, I would consider applying, on his/her behalf, for access to an investigational drug through the federal RTT pathway. (7)	○	○	○	○
I am familiar with the process by which to seek an investigational drug for my patient, through the federal RTT pathway. (3)	○	○	○	○
My clinical practice/ colleagues would be supportive of my choice to seek access to an investigational drug or biologic on behalf of a patient through the federal RTT pathway. (6)	○	○	○	○

Page Break

Q16 Please rate the importance of the following factors in your decision(s) to seek, on behalf of a single patient, non-trial pre-approval access to an investigational drug or biologic outside of a clinical trial setting.

	Very important factor (1)	Moderately important factor (2)	Slight factor (4)	Not a factor (5)
The risks and/or benefits of investigational drugs are not known. (1)	○	○	○	○
I have concerns about legal liability. (2)	○	○	○	○
It is difficult for me to identify investigational drugs/biologics that have potential to help my patient. (3)	○	○	○	○
I do not have enough information to use investigational drugs outside a clinical trial. (4)	○	○	○	○
I do not have enough information about how to seek non-trial pre-approval access. (5)	○	○	○	○
The cost of the drug may not be covered by my patient's insurance (or because of other financial reasons impacting the patient or their family). (7)	○	○	○	○

I may not be paid for my time and effort (or because of other financial reasons impacting me/my institution/my practice). (8)	○	○	○	○
It is difficult to get approval for single patient pre-approval non-trial access to investigational drugs from my institution. (10)	○	○	○	○
I do not have time to support single patient, non-trial pre-approval access requests (12)	○	○	○	○

End of Block: Block 4

Start of Block: Block 5

T9 **Section IV. Your experience with single patient access to investigational drugs, including biologics, outside of a clinical trial setting**

Q17 Please provide the number of patients for whom you have ever sought single patient pre-approval access to an investigational drug or biologic, outside of a clinical trial setting (through either EA or RTT):

- None (1)
- 1 (2)
- 2-5 (3)
- 6-10 (4)
- 10 or more (5)

Q18 Have you ever had a patient that switched to another physician in order to support their desire to seek single patient pre-approval access to an investigational drug or biologic outside a clinical trial (either through your referral or on their own)?

- Yes (1)
- No (2)

End of Block: Block 5

Start of Block: Block 6

Display This Question:

If Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 1

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 2-5

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 6-10

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 10 or more

Q19 If you have ever sought, on behalf of a single patient, access to an investigational drug or biologic outside of a clinical trial setting, please describe the outcome and nature of the process for your most recent experience.

	Did the pharma	Did the FDA allow the	Did you submit	Did the IRB approve or	Did you treat the	Which pathway	Did the FDA

	or biotech company grant access?		request to proceed?			the request to an IRB?		concur with the request?			patient with the investigational drug or biologic?		did you use?		review the request?	
	Yes (1)	No (2)	Yes (1)	No (2)	N/A (3)	Yes (1)	No (2)	Yes (1)	No (2)	N/A (3)	Yes (1)	No (2)	FDA EA (1)	RTT (2)	Yes (1)	No (2)
Single patient non-trial pre-approval access request (most recent experience) (1)																

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Display This Question:

If Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 1

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 2-5

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 6-10

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 10 or more

Q20 For this most recent experience, please rate the importance of the following factors in your decision(s) to seek, on behalf of your patient, non-trial pre-approval access to an investigational drug or biologic outside of a clinical trial setting.

	Very important factor (1)	Moderately important factor (2)	Slight factor (3)	Not a factor (4)
Patient had exhausted all approved therapies (1)	○	○	○	○
No approved therapies were available to treat the patient (2)	○	○	○	○
In my clinical judgment, desired investigational drug was best therapeutic option for patient (3)	○	○	○	○
Parent(s) and/or patient wanted to access investigational drug (4)	○	○	○	○
Other factor; please describe (5)	○	○	○	○

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Display This Question:

If Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 1

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 2-5

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 6-10

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 10 or more

Q21 For this <u>most recent</u> experience, please rate the importance of the following factors in your decision(s) to seek, on behalf of your patient, non-trial pre-approval access to an investigational drug or biologic outside of a clinical trial setting.

	Very important factor (1)	Moderately important factor (2)	Slight factor (3)	Not a factor (4)
Patient was unable to participate in clinical trial for desired investigational drug because he/she did not meet inclusion/exclusion criteria (1)	○	○	○	○
Patient was unable to participate in clinical trial for desired investigational drug because of logistical barriers (4)	○	○	○	○
Patient was unable to participate in clinical trial for desired investigational drug because of financial barriers (5)	○	○	○	○
Patient was unable to participate in clinical trial for desired investigational drug because there were no spots and/or the trial was no longer enrolling (6)	○	○	○	○

Patient was not interested in participating in clinical trial(s) (7)	○	○	○	○

Page Break

Display This Question:

If Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 1

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 2-5

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 6-10

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 10 or more

Q22 Do you believe the patient and/or family benefited from this experience?

- ○ Yes (1)
- ○ No (2)

Display This Question:

If Do you believe the patient and/or family benefited from this experience? = Yes

Q23 How do you believe the patient and/or family benefited from the experience? Please select all that apply.

- ☐ The patient had improvement in overall survival (1)
- ☐ The patient had improvement in quality of life (2)
- ☐ The patient had psychological benefit (e.g., focus on hope) (3)
- ☐ The family had psychological benefit (e.g., focus on hope) (4)
- ☐ Other; please describe (5) _____

Page Break

Display This Question:

If Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 1

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 2-5

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 6-10

Or Please provide the number of patients for whom you have ever sought single patient pre-approval a... = 10 or more

Q24 How did you initially identify the investigational drug/biologic for this single patient non-trial pre-approval access request (your most recent experience)?

- ○ Patient (or family member/guardian) requested it (1)
- ○ Colleague suggested it (2)
- ○ I found it through PubMed (3)
- ○ I found it through clinicaltrials.gov (4)
- ○ I found it through Reagan Udall Navigator (5)
- ○ Other; please describe (6) _____

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Q25 When you sought, on behalf of a single patient, access to an investigational drug or biologic outside of a clinical trial setting, did you receive any assistance from the following? Please check all that apply.

- [ ] Administrative staff within my practice (1)
- [ ] Social worker or patient advocate within my practice (2)
- [ ] Patient advocate outside my practice (3)
- [ ] IRB member or staff at my institution (4)
- [ ] Representative from FDA (5)
- [ ] Representative from manufacturer/ developer of investigational drug or biologic (6)
- [ ] Other; please describe (7) _____
- [ ] I did not receive any support or assistance (8)

Page Break

Q26 Is there anything else you would like to share about your experience(s) with single patient pre-approval non-trial access (compassionate use)? Please do not include any personally identifiable or protected health information about patients.

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End of Block: Block 6

Start of Block: Block 9

T9 **Section V. Survey Submission.**

T10

Please press arrow below to submit your responses. Doing so indicates your consent to participate in this study.

You will be directed to another survey giving you the option of entering the lottery and/or indicating willingness to be contacted about this topic in the future.

End of Block: Block 9