# The "Living With Chronic Neutropenia" Survey: **Experiences and Preferences of People Living With Chronic Neutropenia**

### Background

Chronic neutropenia encompasses different blood disorders characterized by low levels of neutrophils (absolute neutrophil count <1500 for >3 months) that substantially alter the daily lives of affected individuals<sup>1,2</sup>

The current management strategy includes treatment with injectable granulocyte colony-stimulating factor, which may be associated with debilitating side effects, including bone pain and intense fatigue<sup>2-4</sup>

### **Objectives**

To explore experiences of people living with chronic neutropenic disorders and gather their perspective to understand the unmet need and impact on their daily lives through a virtual survey

### Methods



Respondents were recruited—through direct emails to people living with chronic neutropenic disorders and via outreach to chronic neutropenic disorders Facebook groups—and completed a virtual survey comprising 9 questions



The survey included open-ended narrative responses and severity ratings to assess the impact of chronic neutropenic disorders on daily life, document the frequency and severity of infection, and gather opinions of respondents on interest in and barriers to their participation in clinical studies



Respondent	Overview
	•

Respondents 19	
Adults living with chronic neutropenic disorders	Caregiver <sup>a</sup>
	N=20
Age range, y	25–62 <sup>a</sup>
Sex at birth, female, n (%)	15 (75)
Race, n (%)	
White	15 (75)
Black/African	3 (15)
Hispanic/Latino	2 (10)
Diagnosis, n (%)	
Idiopathic	7 (35)
Unknown	4 (20)
Congenital	9 (45)
Cyclic	5 (55)
Persistent	2 (22)
Neither/not sure	2 (22)
Currently prescribed injectables	4 (20)
<sup>a</sup> 1 parent to a female aged 12 years.	



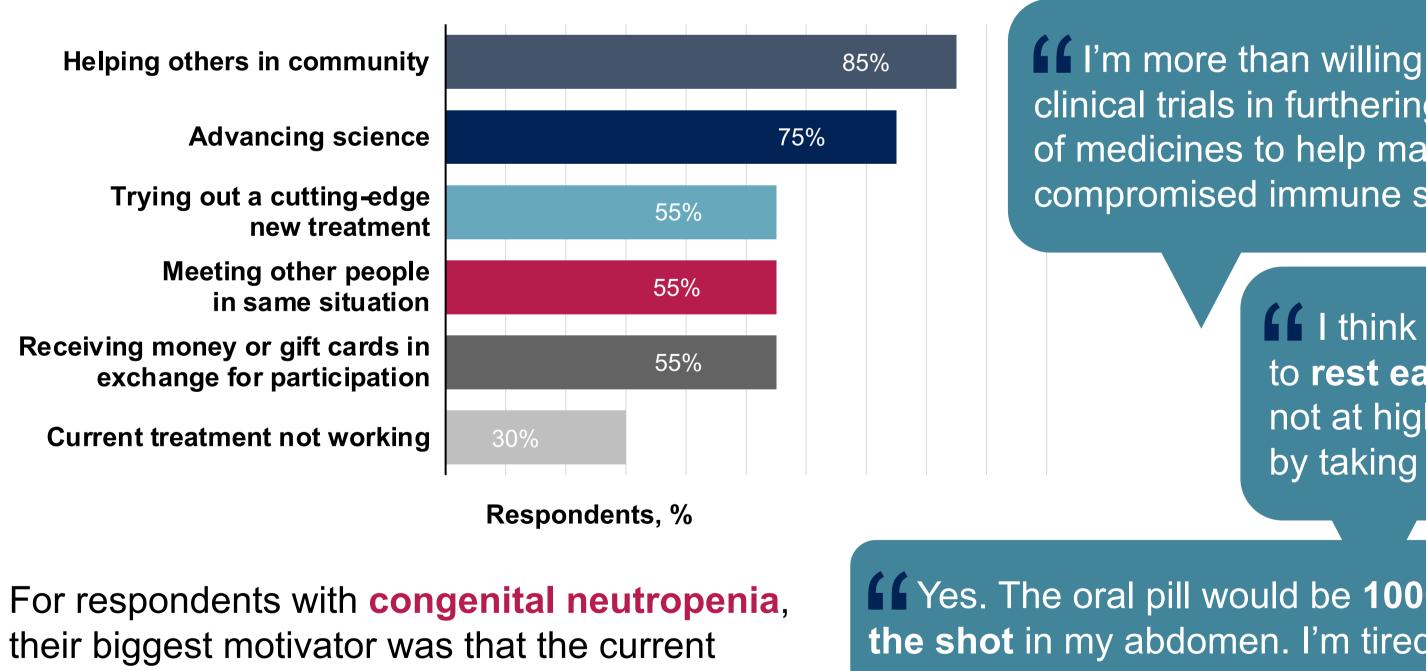


### Conclusions

Anna Ellis, BA; Michele Rhee, MBA, MPH X4 Pharmaceuticals, Inc., Boston, MA, USA

## **Chronic Neutropenic Disorders** Mental and emotional stress (85%) Fatigue

**Results (cont'd) Respondents Reported Multiple Concerns Around Their Psychosocial Experience of Respondents Living With Chronic Neutropenic Disorders Disruption to family** 85% or social life Frequent and/or 60% severe infections 83% My current hematologist is about Disruption to work 55% and school 90 miles from me. He treats my neutropenia like I am a cancer Inability to plan ahead 55% patient because he knows of no Other 45% other way to handle it. I feel like I am ranked this as the biggest Increased hospitalization educating him, but luckily, he impact on their lives 35% due to infection welcomes my input. Bone pain **Respondents**, % COVID anxiety—not everyone is Responses also included fear of infection and isolation, fatigue and other flu-like symptoms, as **cautious** or caring, and it has put a large strain on many relationships. cancer, and frequent blood draws **Risk of potentially I** I'm tired of missing out in life, serious long-term side feeling helpless, feeling like there effects of medications For respondents with **congenital neutropenia**, isn't enough research, knowledge, My concern is that they mostly experienced disruption to work and or treatment options for an infection could school, while those with **idiopathic neutropenia** neutropenic patients. Other take me out. (20%) all experienced disruption to family and social life Participation in Clinical Trials for Chronic Neutropenic Disorders With Oral Medication as a Potential Treatment Option Other respondents had more concerns about participating in clinical trials of respondents answered that they would not be interested in of respondents would be interested in participating in a clinical trial for a new oral medication to treat participating in a clinical trial for a new oral medication to treat chronic neutropenic disorders chronic neutropenic disorders **Biggest Barriers to Participation in Clinical Trials**<sup>a</sup> **Motivation for Participation in Clinical Trials** Travel required (public transport, plane, I'm more than willing to participate in 85% 90% or >3 h by car) clinical trials in furthering the development I would need to know more 75% **Concerns about potential side effects** 75% of medicines to help maintain my about the medication, the compromised immune system. potential risks of it, etc. Unsure of trial requirements and what 55% 70% new treatment signing up for 55% Cost of participating in the trial 55% : would be awesome 55% Days taken from work or school to **rest easier** knowing that I am 5570 I'm concerned about not at high risk of infection just Concerns around catching COVID-19 potential side effects or 50% by taking a single pill. allergic reactions. **Respondents**, % Needing to stop current treatment 50% Yes. The oral pill would be **1000 times better than** Concerns around catching a cold/flu 40% the shot in my abdomen. I'm tired of needles. **Respondents**, % <sup>a</sup>Other responses included overnight stay at a hospital or medical center (25%), potentially receiving a placebo (25%), impact on a care partner (eg, family or friend; 20%), physically going in a hospital medical center (10%) and being the parent of a child with neutropenia (10%) References . Dale DC, et al. Blood. 1993;81(10):2496-2502. 2. Newburger PE, et al. Semin Hematol. 2013;50(3):198-206. 3. Dale DC, et al. Blood Advances. 2022;6(13):3861-3869 4. Anderlini P. Curr Opin Hematol. 2009;16(1):35-40 Disclosures AE is an employee of X4 Pharmaceuticals, Inc, and/or has equity ownership. MR is a former employee of X4 Pharmaceuticals, Inc, and has equity ownership. This study was funded by X4 Pharmaceuticals **Acknowledgements** The authors would like to acknowledge the survey participants, neutropenia Facebook group moderators, and Savvy Cooperative. Authors also acknowledge the medical writing assistance of PRECISIONscientia in Yardley, PA, USA, which was supported financially by X4 Pharmaceuticals in compliance with international Good Publication Practice guidelines.



Despite the health and psychosocial impact of living with chronic neutropenic disorders treatment was not working • The results of this survey demonstrate the major impact of chronic neutropenic disorders on the daily lives of individuals • Results suggest that concerns about adverse events associated with investigational therapies and disruption to respondents' lives should be considered and additional efforts made to help mitigate the risks when designing clinical studies for this population • The outcomes of this survey led to the launch of a second survey and new educational materials, including videos featuring people living with chronic neutropenic disorders, to help highlight the importance of clinical trial participation and ensure requirements are clear and easy to understand

• Additional surveys with larger sample size are needed to expand and confirm these initial results



