

PEOPLE LIVING WITH ALS AND THEIR CAREGIVERS' INPUT INTO DRUG DEVELOPMENT IN EUROPE



M Galvin^{1,2}, O Hardiman^{1,2}, M Heverin¹, C McDermott³, B Charpentier⁴, K Stenson⁵, K Bowyer⁶

¹Academic Unit of Neurology, Trinity College Dublin, Ireland, ² Beaumont Hospital, Dublin, Ireland ³ University of Sheffield, UK ⁴Cytokinetics, South San Francisco, USA; ⁵Biogen, Cambridge, MA, USA, ⁶ Ionis, Carlsbad, CA, USA

Introduction

There is an emerging focus on patient and caregiver engagement in the determination of clinically meaningful outcomes, and for input to key areas of drug development.

The IMPACT European survey of people with amyotrophic lateral sclerosis (ALS) and caregivers is gathering data on the overall burden of disease, psychological distress and the loss of function over the course of the disease.

Objective

To survey ALS patients and caregivers across 9 countries in Europe – UK, Ireland, Germany, France, Spain, Italy, Belgium, Netherlands, Sweden

Findings from the European survey will be viewed in comparison with results from a similar 2017 US survey.

Methods

- The survey questions were used in a previous US survey¹ and adapted for European contexts.
- Recruitment of people with ALS and caregivers (current, former and bereaved) was carried out in each of the participating countries with the partnership of European Network for the Cure of ALS (ENCALS) and advocacy groups in each country.
- GDPR compliant anonymous on-line surveys open from October 2020 with a planned closing date for December 2020.
- Preliminary data are discussed in this presentation
- Ethical approval for this study was received from Trinity College Dublin.

Patient Survey (with assistance as necessary)

- Module 1: ALS Diagnosis and Care
- Module 2: Living with ALS
- Module 3: Approaches to Treating ALS
- Module 4: Background Demographics

Caregiver Survey (current, former, bereaved)

- Module 1: ALS Diagnosis and Care
- Module 2: Living with ALS
- Module 3: Approaches to Treating ALS
- Module 4: Background Demographics
- Module 5: Background and Burden as a Caregiver

Results PRELIMINARY DATA*

* Preliminary Data as on 29th October 2020

FIGURE 1: RESPONDENTS BY SEX

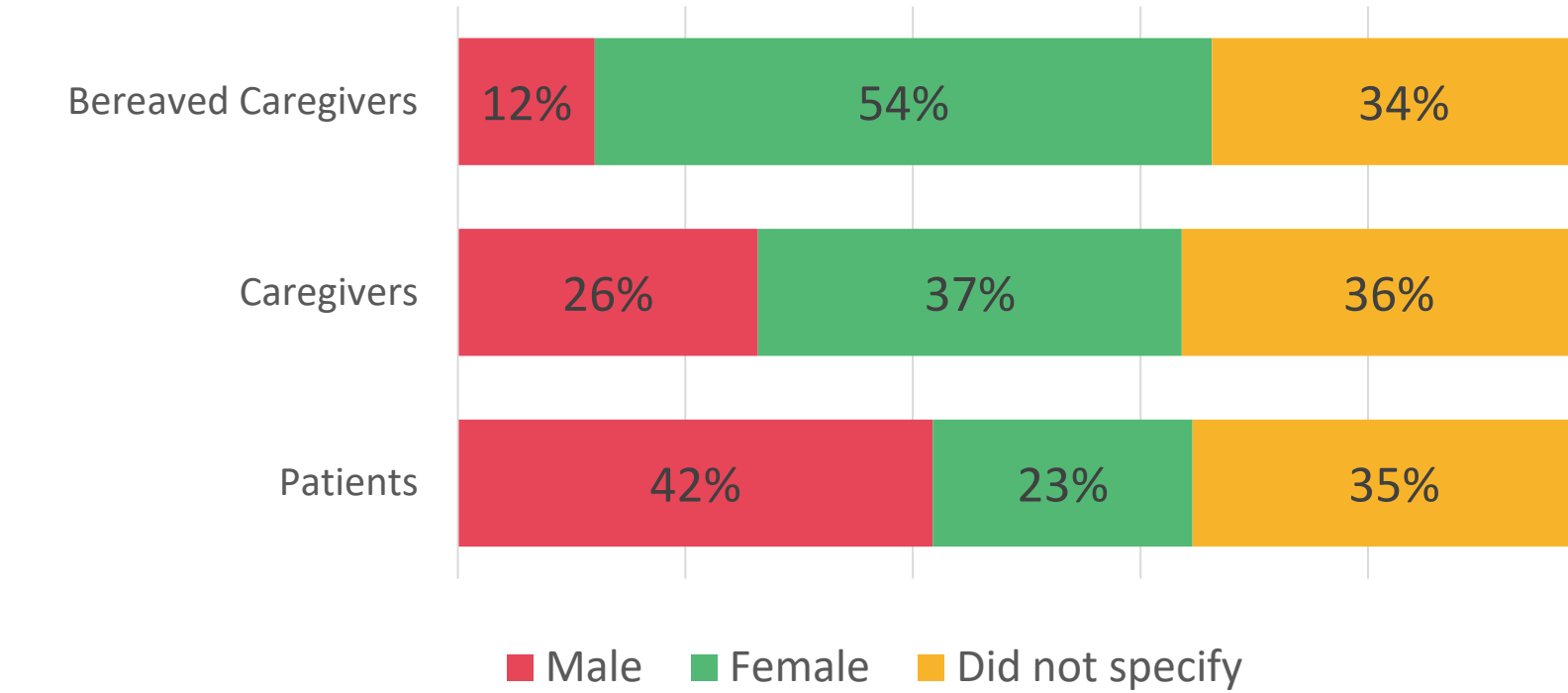
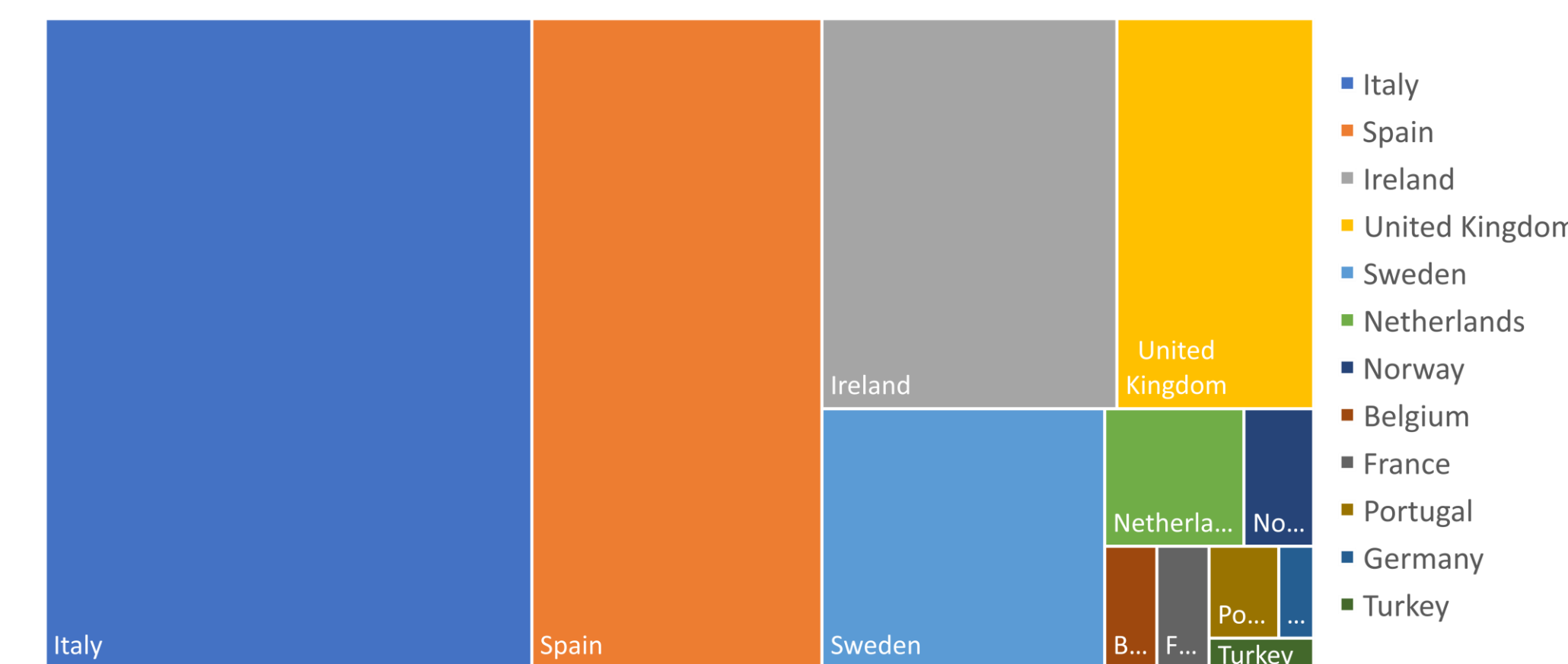


FIGURE 2: RESPONDENTS BY COUNTRY

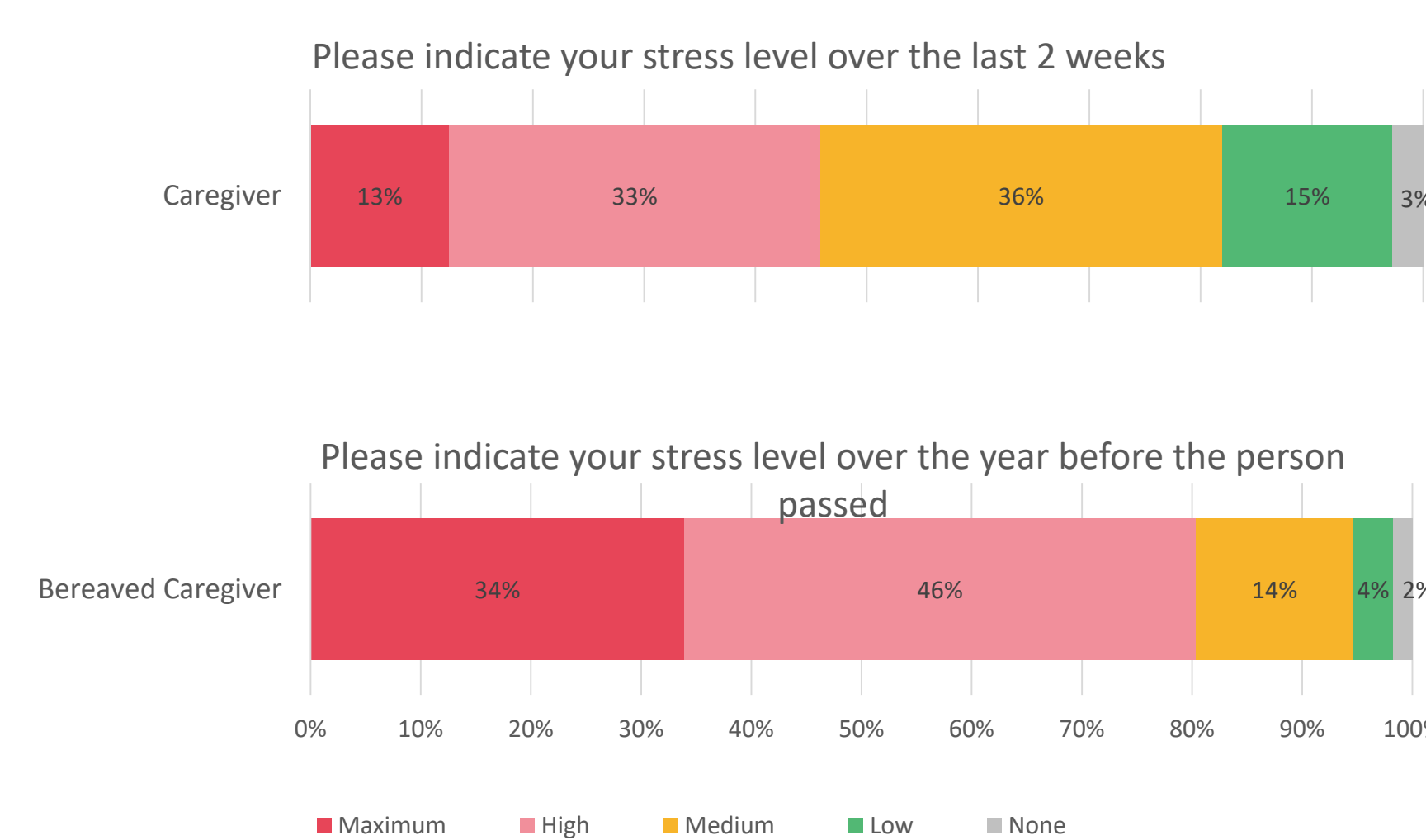


Patients (N=206) Caregivers (N=110) Bereaved Caregivers (N=83) Total (N=399)

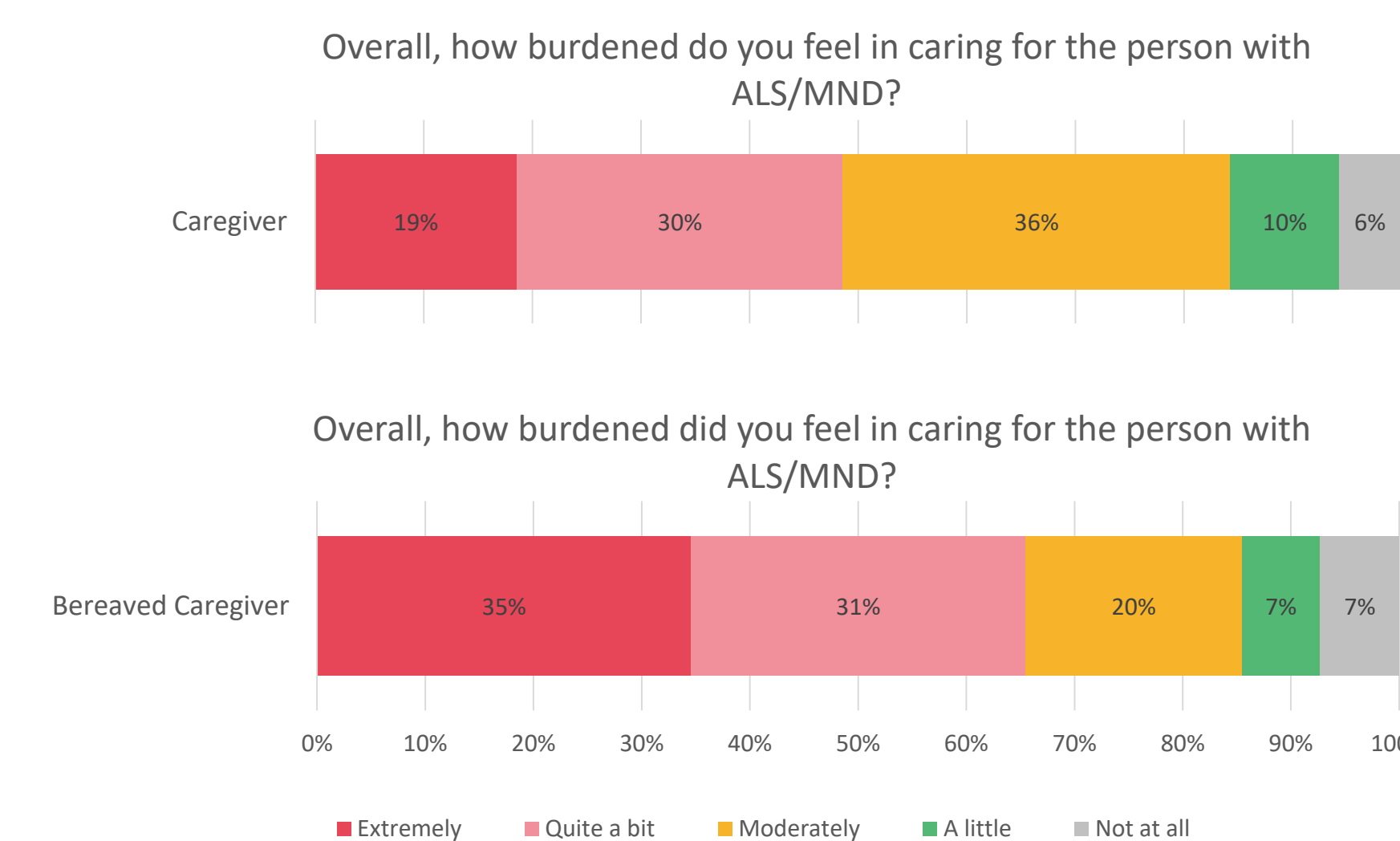
TABLE 1: IN WHICH COUNTRY DO YOU LIVE?

Country	Patients (N=206)		Caregivers (N=110)		Bereaved Caregivers (N=83)		Total (N=399)	
	n	%	n	%	n	%	n	%
Did not specify	73	35%	40	36%	28	34%	141	35%
Italy	63	31%	28	25%	8	10%	99	25%
Spain	17	8%	15	14%	27	33%	59	15%
Ireland	17	8%	13	12%	6	7%	36	9%
United Kingdom	17	8%	4	4%	3	4%	24	6%
Sweden	10	5%	5	5%	8	10%	23	6%
Netherlands	3	1%	2	2%	1	1%	6	2%
Norway	3	1%	0	0%	0	0%	3	1%
Belgium	1	0%	1	1%	0	0%	2	1%
France	0	0%	0	0%	2	2%	2	1%
Portugal	1	0%	1	1%	0	0%	2	1%
Germany	0	0%	1	1%	0	0%	1	0%

FIGURES 3A&B: CAREGIVER STRESS



FIGURES 4A&B: CAREGIVER BURDEN



Discussion and Conclusion

The IMPACT European surveys will be open for completion through to December 2020. There is ongoing monitoring of the national-response numbers and cooperation with local advocacy groups to maintain and encourage participation.

The preliminary data suggest a positive uptake of the surveys and a welcome opportunity to present the voices of patients and caregivers. On survey completion, the analyses of the complete data sets will provide information on issues such as the prevalence of genetic testing, disease symptoms that would preferably be treated, participation in clinical trials and quality of life issues for both people with ALS and their caregivers.

This study provides important information on the burden of disease from the perspectives of people living with ALS and those caring for them in many countries in Europe; this will help to better understand clinical pathways, moments of impact and clinical meaningfulness.

FIGURE 5: WHICH SYMPTOM / FUNCTIONAL IMPAIRMENT DO PATIENTS MOST WANT TREATMENT TO BENEFICIALLY IMPACT?

