

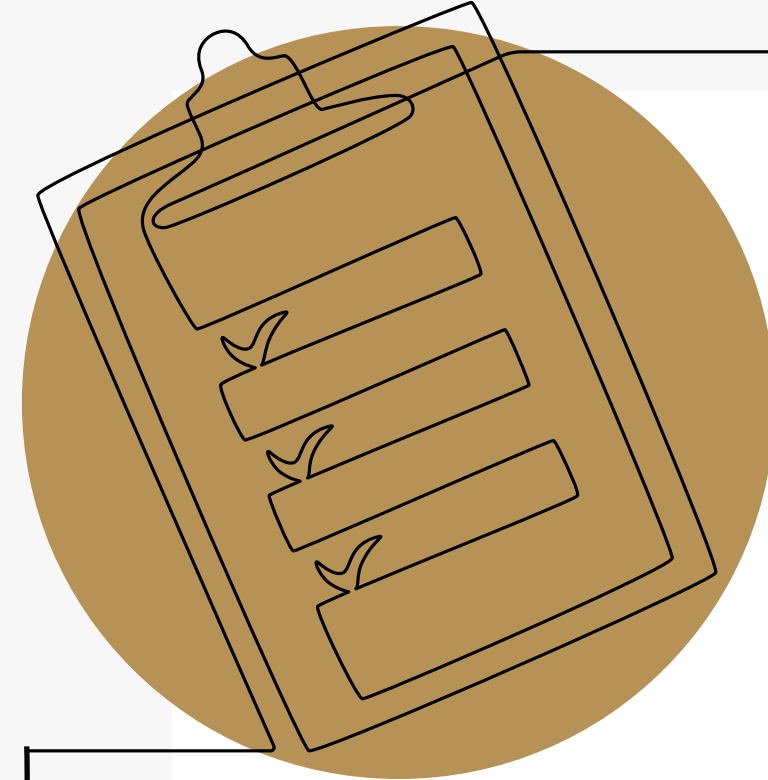
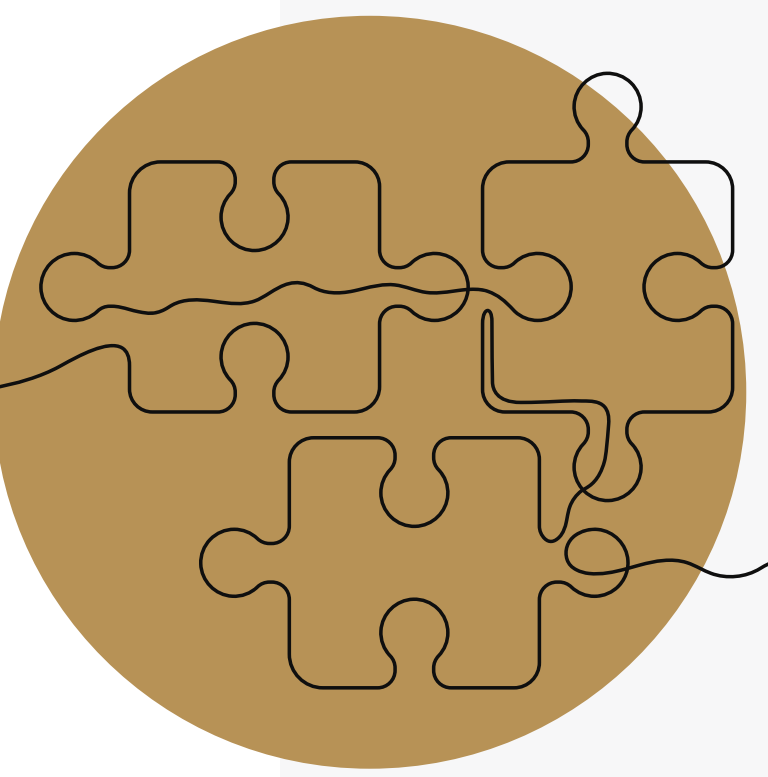
Including People With Associated Conditions in Designing and Reporting Expanded Access Programs: Gaps and Real-World Example

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Introduction

- People living with associated conditions can provide critical input for pharmaceutical companies in the development and implementation of expanded access programs (EAPs), particularly as challenges and new opportunities arise
- Aim: to assess authorship of people living with associated conditions on abstracts pertaining to EAPs and provide a real-world example of a pharmaceutical company that included people living with amyotrophic lateral sclerosis (PLWALS) and their caregivers into its publication strategy

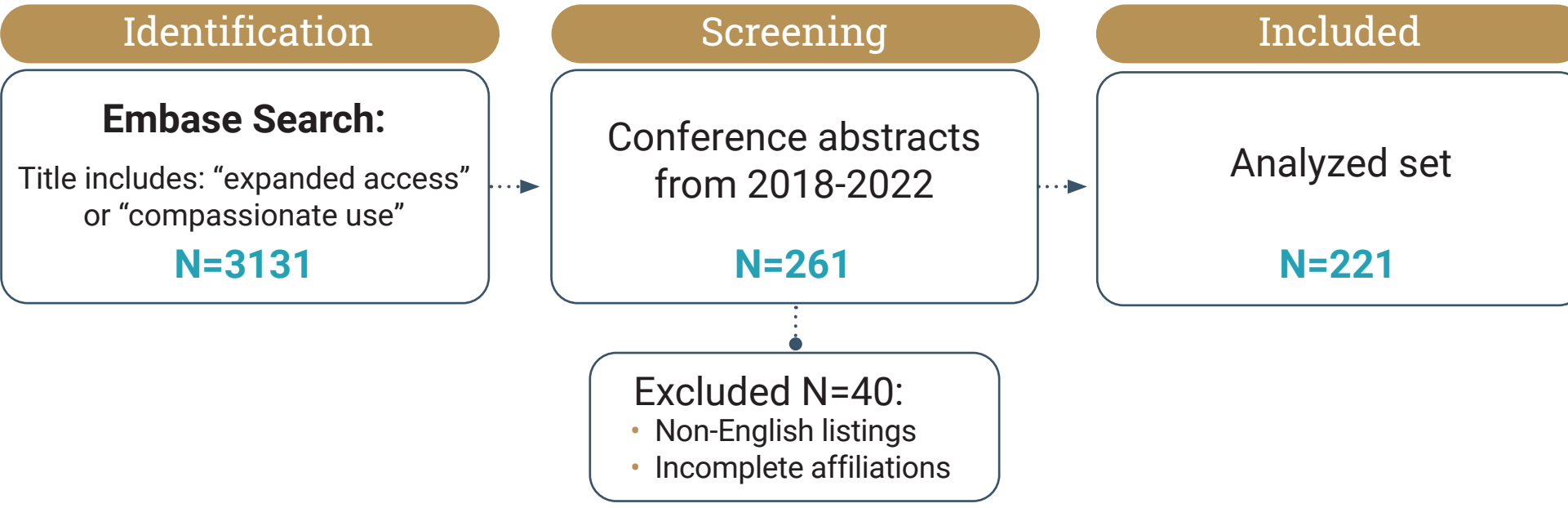


Methods

- Performed an Embase search for English-language conference abstracts from 2018-2022 with the terms “expanded access” or “compassionate use” in the title
- Reviewed affiliations for authors and characterized as: pharmaceutical/industry, hospital/university/academic, people living with associated conditions/patient advocacy, and other (eg, governments, military, or independent companies)

Results

Only a small number of congress abstracts on EAPs included author representation of people living with the associated condition



Authorship within the Embase-analyzed set (N=221)



- 6 abstracts had representation of people living with associated conditions
 - 2 abstracts were authored by patient advocacy groups; 1 from I AM ALS (4 authors) and 1 from Kids v Cancer (2 authors)
 - 4 abstracts included companies that provide access to EAPs (n=3 MyTomorrow, n=3; Early Access Care, n=1)

Limitations

- Limitations of this study are that it only evaluated Embase-indexed abstracts and would categorize authors who are living with the associated conditions who are also affiliated with industry/hospitals/universities as being industry/academic and not patient advocacy/people living with associated conditions

Case Study

Prioritizing involvement of people living with the associated condition led to strong and successful collaboration for an EAP in amyotrophic lateral sclerosis (ALS), from study design through publication

1. Design

EAP development:

- Was performed in collaboration with PLWALS and caregivers
- Was achieved via regular calls on study design, implementation, and ongoing progress
- Resulted in the largest ALS EAP to date, providing PLWALS with limited treatment options access to an investigational therapy

Learning

PLWALS were integral to the EAP success, providing feedback on feasibility, communication in a complex space, and ongoing implementation



2. Strategy

PLWALS authors were considered in publication strategy decisions
Adjustments were made to ensure complete collaboration throughout the process

- Speaking about science at appropriate level
- Avoiding use of inaccessible systems

Learning

Publication review will be more challenging within publication management platforms, so plan for reviews and approvals via calls and email, with documentation



3. Execution

Collaboration ensured appropriate language was used to maintain people-centricity
Approach involved engaging poster design and QR links to supplementary material including:

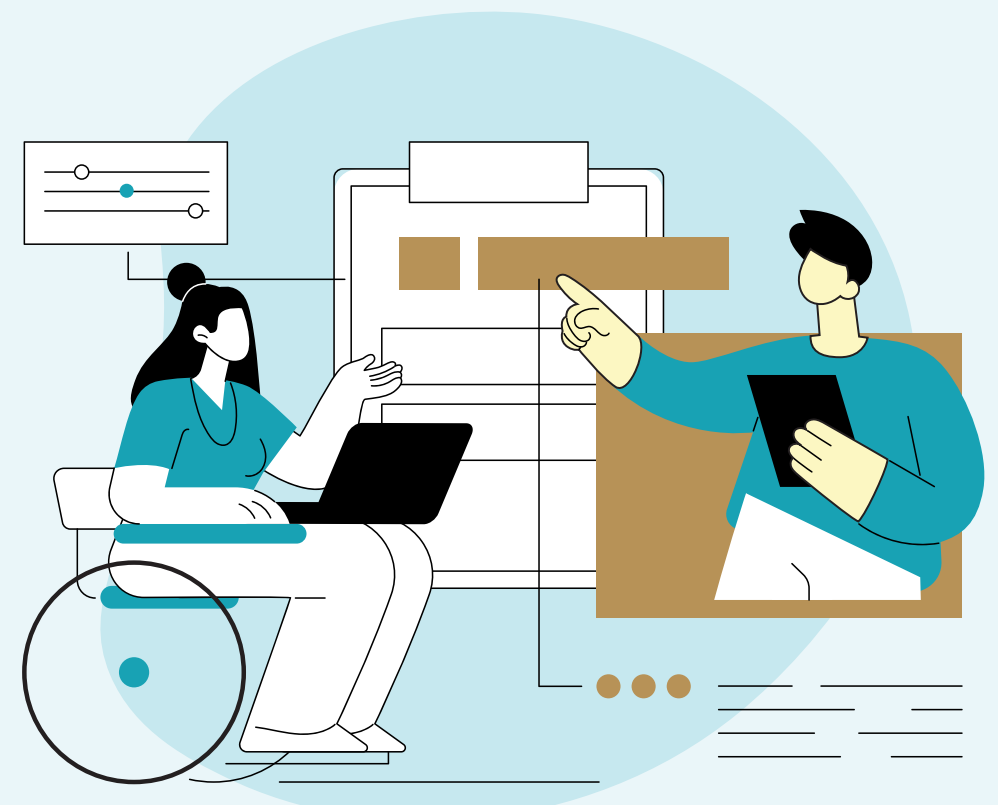
- Audio of 2 PLWALS authors hosted on Vimeo with closed captioning
- Audio of a PLWALS author hosted on the conference portal

Learning

Present material in an accessible manner, using inclusive language, easy-to-interpret graphics, and additional tools such as captioning for audio



View poster examples and listen to PLWALS authors Sandy Morris and Philip Green share their advice on EAP design



4. Outcomes

The material was presented at 2 conferences
1 PLWALS presentation won the “People’s Choice Poster” award at that conference
Posters were made freely available with presentation web hosting

Learning

PLWALS and those with other diseases often cannot access publications, particularly congress material, even when participating as an author

Conclusions

- People living with associated conditions rarely see representation in conference abstracts pertaining to EAPs, but this people-centric publication plan led to broad audience reach and acclaim
- In this plan, PLWALS made important improvements to the design, conduct, and publication of the EAP, highlighting the importance of including these stakeholders’ viewpoints in all parts of the EAP process

Disclosures

- TL, JM, LP, and BW are employees of PRECISIONscientia, and have been contracted by Amylyx Pharmaceuticals, Inc. to provide medical writing, planning, and strategic support. JK, KHB, JC, JBK, MM, and JT are full time employees of and have stock option ownership of Amylyx Pharmaceuticals, Inc.

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