

Patients and caregivers' core aspirations to maximize the attractiveness of **ASPIRE** - a phase 2b trial to investigate the disease modifying potential of buloxibutid in IPF



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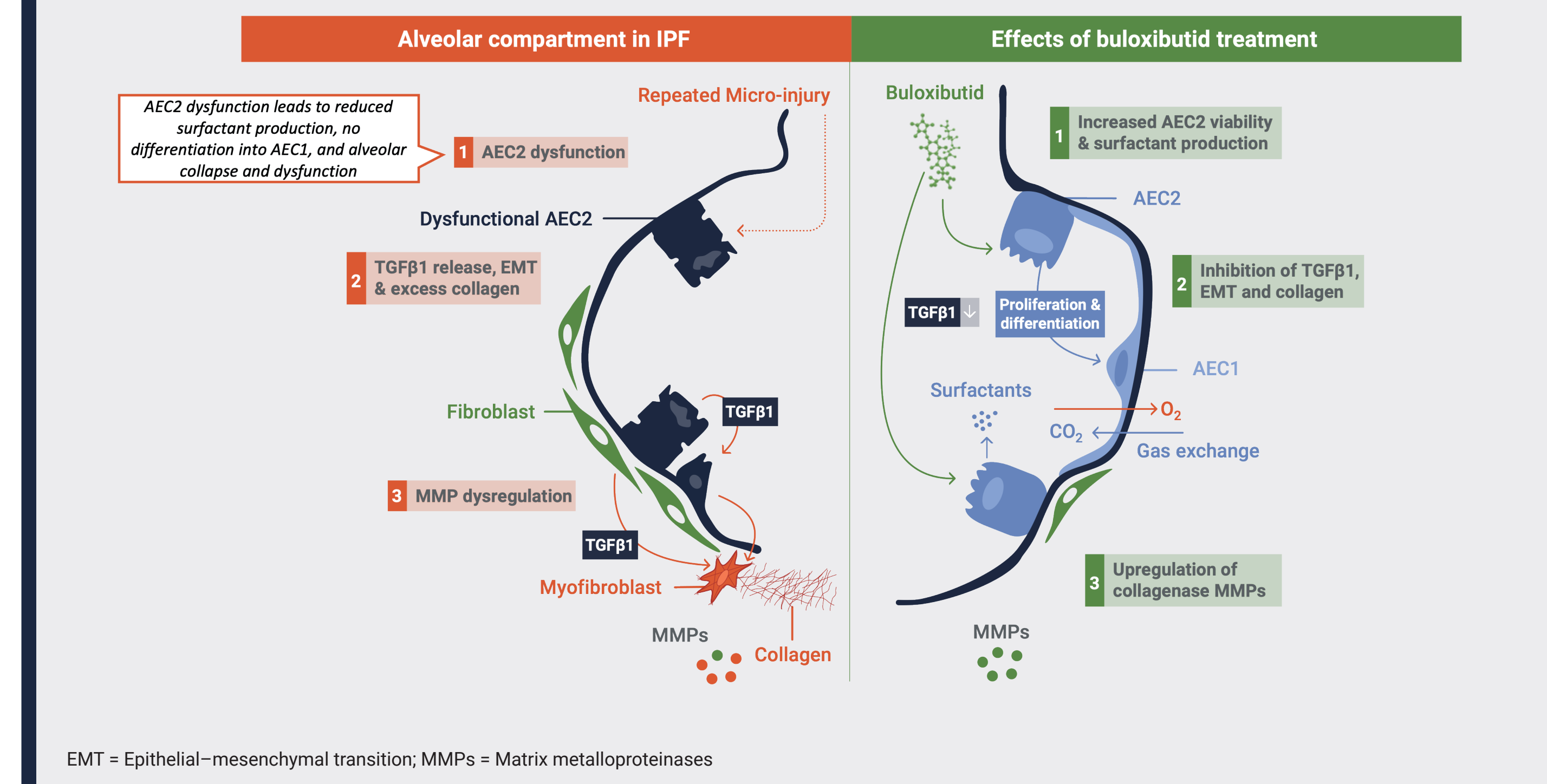
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Background and rationale

The unmet medical need in idiopathic pulmonary fibrosis (IPF) remains monumental with multiple trials failing to show stabilization and improvement. Additionally, many clinical trials fail to recruit enough participants or suffer high dropout rates, increasing the hurdles for developing new and effective therapies.¹

Buloxibutid (C21) is a novel oral, selective angiotensin II type 2 (AT2) receptor agonist with a disease modifying potential in IPF. Buloxibutid drives an upstream pathway to improve alveolar epithelial type 2 cell (AEC2) function, triggering a cascade of anti-fibrotic activity, and resolving disease-associated vascular remodeling.

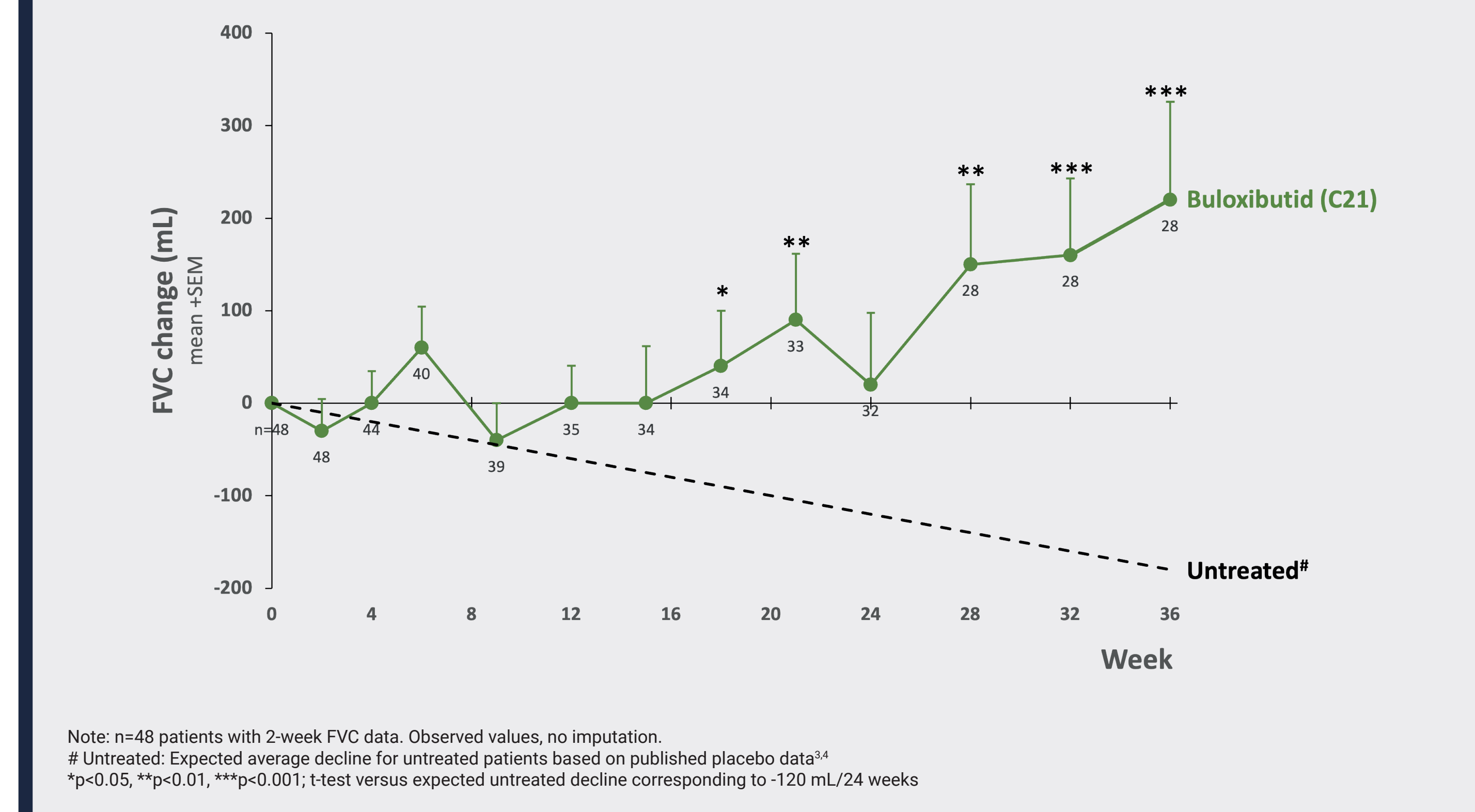
Buloxibutid is an oral, selective AT2R agonist driving tissue repair in IPF



Buloxibutid was found to stabilize and improve lung function over 36 weeks in the phase 2a AIR trial. The safety profile shows excellent gastrointestinal tolerability and no treatment related serious adverse events. The final results from the AIR trial were disclosed during the oral, late-breaking presentation at the ATS congress earlier this year.

Learning from the AIR trial and recent publications, Vicore recognizes that to meet patients' needs and priorities, and to ensure that trial participation is attractive and convenient, increased patient involvement in trial design and set-up is required.^{2,5}

Buloxibutid stabilizes and improves lung function over 36 weeks²



Methods

To increase the probability of success of ASPIRE, a 52-week, randomized, double-blind, placebo-controlled, phase 2b trial of buloxibutid, early patient involvement is secured by the establishment of an advisory panel consisting of six patients and two caregivers from the United Kingdom and the United States. Multiple interactions are planned during the set-up and conduct of the trial. The first interaction included a two-hour, semi-structured panel discussion followed by five individual interview sessions. Qualitative feedback was sought on the participants' clinical research experience and the ASPIRE trial protocol and patient-facing materials.

Results

The six patient panellists were 61-80 years of age, predominantly males (83%), with an IPF diagnosis for 0-5 years. All were on approved IPF therapy and 50% had previous clinical trial experience. The two caregiver panellists were 71-80 years of age and females. The feedback from the first interaction fell into three main categories; patient first, patient feedback and promises fulfilled – the 3 PFs.

In line with the feedback received, in the ASPIRE trial, patients will continue standard-of-care treatment and a 2:1 allocation to buloxibutid versus placebo increases the chance of receiving active treatment. Trial visits are partly decentralized, i.e., several visits are conducted as phone or video call from patients' home and on-site visit frequency kept to a minimum, to reduce travel burden. Patient and caregiver input will continue through the panel during trial execution, including feedback on enrollment, potential amendments, and trial procedures. Patient-involvement activity will be documented and published to enable sharing of experiences and learnings.

The 3 PFs and qualitative feedback*

- Patient First**
 - Importance of **understandable, comprehensive and transparent trial communication to trial participants and caregivers**, including trial design, procedures, potential benefits, side effects and individual trial progress.
 - Consider **trial participant and caregiver needs and preferences** including compensation and support services, visit schedule flexibility, home-based monitoring and efficient coordination at trial sites to minimize waiting times.
- Patient Feedback**
 - Importance of **collecting qualitative and quantitative patient feedback** throughout trial planning, conduct and reporting.
 - Use **multiple sources** for patient feedback e.g., through patient organizations and support networks, trial-specific panels and trial participants.
- Promises Fulfilled**
 - Being able to fulfill promises** is a key motivation for trial retention and further engagement.
 - Realistic expectation management** can be achieved by including patients and caregivers in the process.

* Documented qualitative feedback collected during the first patient and caregiver panel interaction for ASPIRE.



Discussion

Increased involvement of patients and caregivers in the design of clinical trials has been strongly advocated, and is also encouraged in the International Council For Harmonisation (ICH) guidelines. Aspects from potential participants will likely increase trust in the trial, facilitate enrollment and promote adherence, since they have valuable insights into whether scheduled procedures may be overly burdensome and lead to early dropouts.⁶ The importance of anchoring IPF trial endpoints on real patient experiences - namely, how they feel, function, and survive - is also pointed out by multidisciplinary stakeholders (patients, investigators, and regulatory representatives) in a recent publication.⁷

Conclusions

- IPF patients need access to new improved treatments which requires scientifically rigorous trials that are holistic and patient centric.
- ASPIRE aims to achieve these through continuously listening to patients and caregivers with an ambition to increase trial participant empowerment.
- Vicore is in the forefront by committing the 3 PFs to guide the design, conduct and reporting of the ASPIRE trial.



Acknowledgements

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¹ Desai M. Recruitment and retention of participants in clinical studies: Critical issues and challenges. *Perspect Clin Res.* 2020 Apr-Jun;11(2):51-53.

² Ganslandt C et al. *Am J Respir Crit Care Med* 2024; 209: A1055

³ Noble et al. *Eur Respir J.* 2016 Jan; 47(1): 243-253

⁴ Richeldi et al. *Engl J Med* 2014; 370:2071-2082

⁵ Jones S et al. *ERJ Open Research* 2022; 9: 006602:2022

⁶ ICH (2021). ICH Guideline E8(R1): General Considerations For Clinical Studies.

⁷ Raghu G et al. *Am J Respir Crit Care Med* 2019, Iss 6, pp 647-669, Mar 15, 2024

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