# A Qualitative Interview Study to Evaluate Single-tablet Combination **Therapy (STCT) within a Phase 3 Pulmonary Arterial Hypertension** (PAH) Clinical Trial – Interim Analysis

Davis S<sup>1</sup>, Randall JA<sup>2</sup>, Linder J<sup>3</sup>, Rafalski J<sup>4</sup>, Hughes M<sup>2</sup>, Agustin Rodriguez A<sup>5</sup>, Friberg M<sup>6</sup>, Burbridge C<sup>2</sup>

<sup>1</sup>Janssen Global Services, LLC, Horsham, PA, USA; <sup>2</sup>Clinical Outcomes Solutions, Chicago, IL, USA; <sup>5</sup>Janssen Research and Development, LLC, Titusville, NJ, USA; <sup>1</sup>Janssen Research and Research an <sup>6</sup>Actelion Pharmaceuticals Ltd, a Janssen Pharmaceutical Company of Johnson & Johnson, Allschwil, Switzerland

#### Introduction

- Pulmonary arterial hypertension (PAH) is a rare, chronic, and progressive disease leading to right ventricular (RV) failure and death<sup>1–3</sup>
- PAH should be treated with double/triple therapy in most cases, requiring multiple tablets.<sup>1</sup> Patients often have co-morbidities and can be on multiple treatments,<sup>4</sup> which can impact adherence to medication<sup>5–7</sup>
- A single-tablet combination therapy (STCT) of macitentan 10 mg and tadalafil 40 mg compared with corresponding monotherapies is being evaluated in a Phase 3 trial (A DUE, NCT03904693, AC-077A301): patients received 4 tablets during the double-blind period to maintain blinding (Various arms: 1 STCT + 3 placebos; 1 macitentan 10 mg + 3 placebos; 2 tadalafil 20 mg + 2 placebos) and 1 tablet (STCT) during open-label treatment
- During the clinical trial double-blind portion STCT led to a highly significant and marked improvement in primary endpoint pulmonary vascular resistance (PVR)-reduction vs macitentan and tadalafil monotherapies<sup>8</sup>
- A trend for clinically relevant improvement in 6 Minute Walk Test in favor of STCT was observed<sup>8</sup>
- Interviews during the ongoing open-label extension are being conducted to explore patients' and clinicians' experience with the STCT, specifically the adherence and convenience of this medication

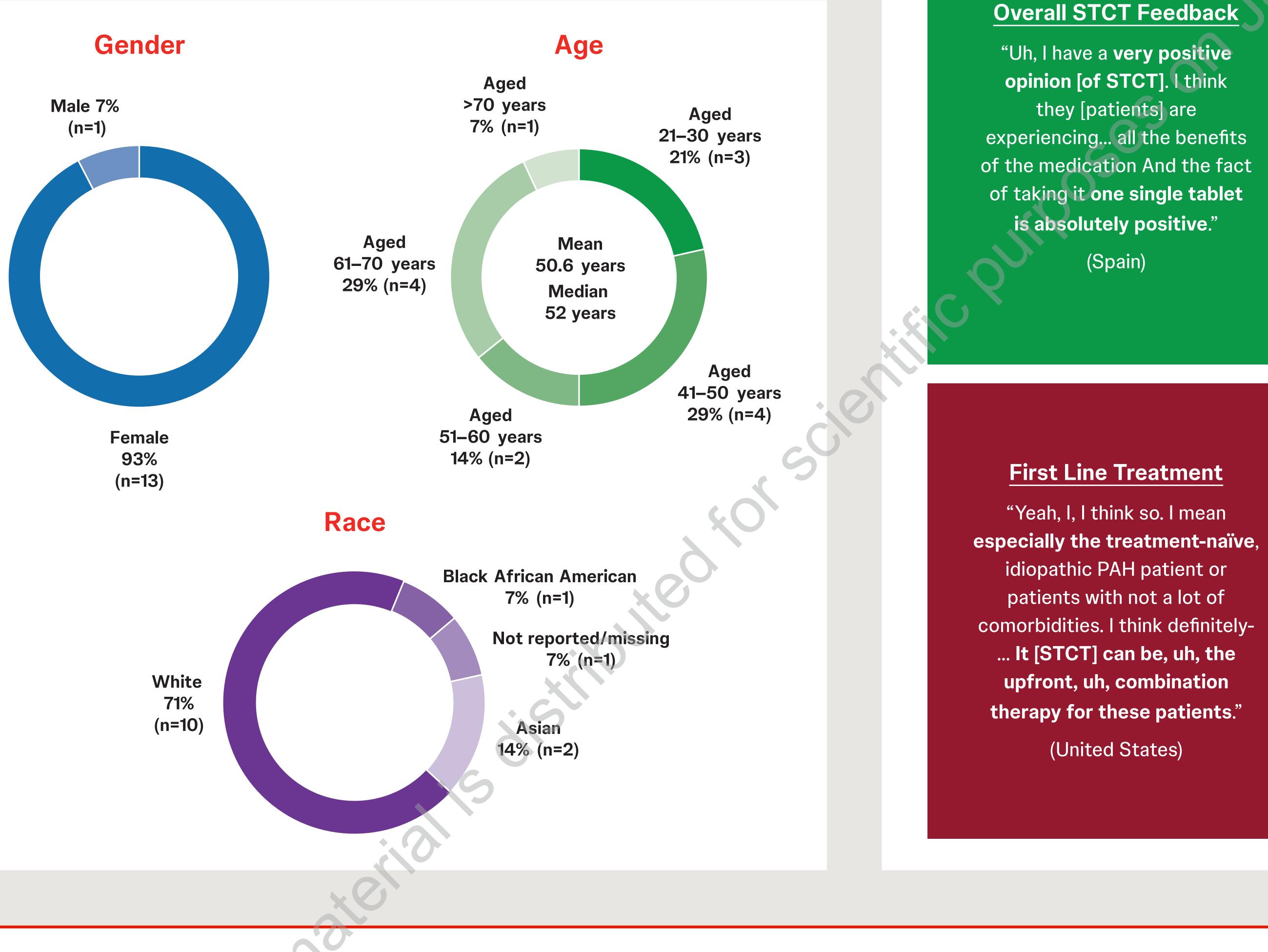
#### Methods

- Qualitative, one-on-one, semi-structured web-assisted interviews are being conducted across multiple countries with A DUE trial patients and clinicians
- Interviews asked both patients and clinicians about their experience with the STCT, adherence, convenience, and the impact of a reduction in pill count. In addition, clinicians also discussed STCT in relation to treatment-naïve patients and the ESC/ERS guidelines<sup>1</sup>
- All data was analyzed using Thematic analysis<sup>9</sup> which was conducted using NVivo v14
- To be eligible for the study, patients had to be currently receiving the STCT during the open-label treatment period of the clinical trial, or the continuous access program, or recently stopped taking the STCT (in the last 2 weeks). Patients had to have taken the STCT for at least 6 months prior to the interviews
- Clinicians are investigators from the A DUE clinical study sites. There were no additional clinician eligibility requirements
- A total of 9 countries (Brazil, Germany, Mexico, Poland, South Africa, Spain, Taiwan, Turkey, United States) have been identified for participation in this study
- This report is based on interim data and analysis from 14 patient and 13 clinician interviews conducted up to the 13th of October 2023 (Germany: 1 clinician; Mexico: 1 clinician; Poland: 1 patient, 1 clinician; South Africa: 2 patients, 1 clinician; Spain: 2 patients, 1 clinician; Taiwan: 2 patients, 1 clinician; Turkey: 2 patients, 1 clinician; and US: 5 patients, 4 clinicians)
- Recruitment and interviews are ongoing across multiple countries, due to complete in Q1 2024

#### Results

- compared with STCT
- managing multiple tablets

#### Patient Demographics, N=14



# **REFERENCES:**

• All 27 participants (14 patients and 13 clinicians) provided **positive feedback on the STCT** and preferred the single tablet in the open-label extension to the four tablets in the doubleblind period **regardless of the country** or treatment in the double-blind period

• Patients stated the **STCT** was convenient, aided adherence, and had a positive impact on their day-to-day lives. Patients discussed how taking more tablets made them feel "sicker"

• Patients noted the STCT improved their psychological well-being and reduced the stress of

• Clinicians noted that the typically high pill burden in PAH can cause emotional distress in their patients, whereas patients had higher treatment satisfaction with the STCT

• Clinicians predict the STCT will be well received in clinical practice and endorsed prescribing STCT for their patients, with the majority also endorsing use for treatment-naïve patients

#### **Clinician Profiles**

- each month
- with PAH per month
- Organization functional class evaluation categories

**Clinician Quotes** 

1. Humbert M, et al. Eur Heart J. 2022;43:3618–731. 2. Montani D, et al. Circulation. 2010;122:164–72. 4. Suarez JA, et al. Circulation. 2010;122:164–72. 4. Su 7. Qadus S, et al. Am J Cardiovasc Drugs. 2023;23:19–33. 8. Chin K, et al. Presented at ACC New Orleans, 6 March 2023. 9. Braun V & Clarke V. Qual Research Psychol. 2006;3:77–101.

 Most of the clinicians were cardiologists who worked in multi-disciplinary hospitals, and all had at least 5–9 years of experience working in PAH, seeing at least 5–10 PAH patients

- Several clinicians had many more years of experience and saw a greater number of patients

All clinicians saw patients with a similar spread of disease severities based on World Health

#### Adherence

"I believe that it [STCT] will improve adherence of pat- for the patient, and will make their life easier." (Germany)

#### **Reduction in Pill Burden**

"If you **reduce a pill burden** in a patient who's taking at baseline, you know, five or six tablets, yeah, uh, **it makes a** big difference."

(South Africa)

#### Suitable for all Patients

"I think it will be **absolutely** suitable [for everyone], and it will be, uh, an interesting improvement in...if we can offer them a combination in a single tablet, this is... this is positive, of course."

(Spain)

#### **Patient Quotes**

<b>Overall STCT Feedback</b>	
"Yeah. It's really nice to take only one tablet- Yes. So many times, I just wanted to tell the doctors like, you know, "I don't want to take all these tablets now." (South Africa)	"lt's g pil te tha
<u>Convenience</u> "I would honestly be ecstatic [to	

continue taking the single tablet] Um, a lot easier to fit just one pill for something, um, into my rotation or my schedule. Um, I wish I could do that for all of my stuff."

(United States)

#### **Better Mental Health**

"Um, every pill makes me a little bit more sad that, you know, I'm, I'm sick, and it's just a re-, a daily reminder that I'm sick, and having fewer pills just makes me feel better about myself." (United States)

#### <u>Convenience</u>

"Um, it, it **simplifies things**. It simplifies my life. It is, um, much more convenient, and, um, it **gives me confidence** that I'm doing something good for myself."

(United States)

a good thing. Psychologically it's a od thing. Uh... now I'm taking one instead of three or four. The **brain** Is you that it's... Well... it tells you a**t it's better**. I feel better. I'll tell you that. It's better for me."

"The fewer the tablets, it's always better [for adherence]."

"For me, it's easy. I have already a routine and I have... I wake up, have breakfast, my pill, you know? Once I had my routine stablished, I already... And as it is working fine, I don't forget to take it. That's for sure."

#### **Better Mental Health**

(Turkey)

#### Adherence

(United States)

#### **Reduction in Pill Burden**

.. it's better to take one, right? Rat han 4 and having to remember the ings. Because as I mentioned, even go out or meet friends or family, . ou don't have to remember and keep track of taking the pill." (Poland)

#### Adherence

(Spain)

### Conclusions

Interim analysis suggests that the STCT was well received by patients and clinicians regardless of the country



The qualitative insights from this study show, directly from the patients living with PAH, that reducing the number of tablets needed to treat PAH would be highly valued by patients and would have a **positive effect on** patients' well-being



The participants in this study highlighted how the STCT reduced pill burden, minimized stress, improved adherence to treatment and simplified the treatment regimen, which could improve patient outcomes and healthrelated quality of life (HRQoL)



These findings align with previous research that has also provided strong evidence that reducing the tablet count and frequency has a major impact on patient adherence to PAH therapies, and subsequently clinical outcomes<sup>6,7</sup>

## Limitations



The study has been conducted on limited interim data, and interviews are ongoing

#### Acknowledgments

J.A.R., J.R., M.H., and C.B., are employees of Clinical Outcomes Solutions, a health research consultancy paid to conduct this research by Janssen Pharmaceutical companies of Johnson & Johnson. S.D., J.L., A.A.R., M.F., are employees of Janssen Pharmaceutical companies of Johnson & Johnson and own shares of stock/stock options in Johnson & Johnson.

#### Disclosures

This research was funded by Actelion Pharmaceuticals Ltd, a Janssen Pharmaceutical Company of Johnson & Johnson.