unfavourable tuberculosis treatment outcome vary between adolescents (10–17 years) and young adults (18–24 years), and the extent to which they are included in treatment support strategies.

**Methods** A national retrospective cohort study was conducted using Brazilian tuberculosis registry data to investigate the predictors of unfavourable treatment outcome for young people with tuberculosis. Persons between 10–24 years, with newly diagnosed tuberculosis between January 2015 and December 2018, were included. Unfavourable outcomes were defined as loss to follow-up, treatment failure, change in treatment, death. Favourable outcomes were defined as treatment completion or cure. Factors associated with unfavourable treatment outcome were compared between adolescents and young adults, using complete case and missing indicator multiple logistic regression models.

**Results** 41,870 young people were included, 7,024 (17%) experienced unfavourable treatment outcomes.

5,869 (14%) were lost to follow-up; of which 86% were young adults, 72% male, 73% identifying with black or brown skin colour, 67% had low educational attainment and 9% diagnosed with HIV/AIDS. HIV/AIDS ($OR_{adj}$4.52;95% CI:2.85–7.17), drug use ($OR_{adj}$3.66;95% CI:2.36–5.70), identifying with black skin colour ($OR_{adj}$2.07;95% CI:1.44–2.98) or low educational attainment ($OR_{adj}$2.06;95% CI:1.64–2.59) were most strongly associated with unfavourable outcome in adolescents, as compared to young adults. Conversely, deprivation of liberty was only protective for young adults ($OR_{adj}$0.56;95% CI:0.48–0.66). Adolescents and young adults with tuberculosis had similarly low uptake of treatment supervision (52% vs. 52%; p=0.92), however adolescents were more likely to receive governmental cash transfers compared to young adults (17% vs. 8%; p<0.05).

**Conclusions** HIV/AIDS, drug use, race and educational attainment are the strongest independent predictors of unfavourable outcome for both adolescents and young adults with tuberculosis in Brazil. Greater efforts are needed to engage vulnerable young people with tuberculosis in treatment support strategies, including treatment supervision and governmental cash transfers.

## S11 LONG-TERM FOLLOW-UP OF THE PHASE 1 START TRIAL OF ONASEMNOGENE ABEPARVOVEC GENE THERAPY IN SPINAL MUSCULAR ATROPHY TYPE 1

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**Introduction and Objective** Onasemnogene abeparvovec (formerly AVXS-101) is designed to address the genetic root cause of spinal muscular atrophy type 1 (SMA1). In the phase 1 trial (START; NCT02122952), patients who received a one-time (proposed therapeutic dose) infusion (n=12) demonstrated significantly improved outcomes versus untreated natural history. Here, we evaluate long-term safety in patients previously treated in START and long-term efficacy in patients from both Cohorts. START patients could electively enroll into a LTFU study (NCT03421977).

**Methods** Primary objective: long-term safety. Patients have annual visits (5 years) followed by annual phone contact (additional 10 years). Assessments include medical history/record review, physical examination, clinical laboratory evaluation, pulmonary assessments, and milestone maintenance.

**Results** As of 31 Dec 2019, 13 patients (low dose, n=3; therapeutic dose, n=10) were enrolled. The oldest patients were aged 6.2 (low dose) and 5.6 (therapeutic dose) years. All patients who received the therapeutic dose have survived and are free of permanent ventilation (mean [range] age at last data cut: 4.8 [4.3–5.6] years; mean [range] time since dosing: 4.5 [4.1–5.2] years). These patients have either maintained all previously attained milestones or gained new milestones; 2 patients have newly achieved standing with assistance while not receiving concomitant survival motor neuron 2 protein (*SMN2*) upregulating therapy at any point. Of the 10 enrolled patients who received therapeutic dose, 6 did not require regular, daily respiratory support more than 4 years after dosing. Additionally, 6 have never received concomitant *SMN2* upregulating therapy. No new treatment-related adverse events (AEs) were reported. Onasemnogene abeparvovec has been associated with transient, manageable, and subacute AEs. During LTFU, no AEs of special interest have been reported to date, specifically none associated with liver enzyme elevations, haematology values, new malignancies or autoimmune disorders. Serious AEs were reported in 8/13 (61.5%) patients; however, no serious AEs were considered related to treatment or lead to study discontinuation supporting a favorable risk–benefit profile.

**Conclusions** Onasemnogene abeparvovec shows a favorable risk–benefit profile, and continues to demonstrate efficacy with new milestone developments.

## S12 ONASEMNOGENE ABEPARVOVEC GENE THERAPY FOR SPINAL MUSCULAR ATROPHY TYPE 1: PHASE 3 STUDY (STR1VE-US)

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**Introduction and Objective** Onasemnogene abeparvovec (formerly AVXS-101), is designed to address the genetic root cause of spinal muscular atrophy (SMA), survival motor neuron 1 gene (*SMN1*) deletion/mutation. Here, we evaluate final data from STR1VE-US (NCT03306277), a multicenter, open-