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Title: Results of a phase 2b multi-center, randomized, double-blind, placebo-controlled study of an RNAi therapeutic, ALN-RSV01, in respiratory syncytial virus (RSV)-infected lung transplant patients

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Body: RSV infection after lung transplantation is an independent risk factor for the development of bronchiolitis obliterans syndrome (BOS). ALN-RSV01 is a small interfering RNA targeting the RSV nucleocapsid gene that is critical for viral replication. Previously, we performed a Phase 2 randomized, double-blind, placebo (PBO)-controlled trial in 24 RSV-infected lung transplant patients administering aerosolized ALN-RSV01 or PBO for 3 days. The primary endpoint of safety and tolerability was attained. In addition, there was a significant reduction in the secondary endpoints of incidence of new or progressive BOS at day 90 (p=0.027) and patient's symptom scores in the ALN-RSV01 group compared to PBO. To extend these results, we performed a Phase 2b multi-center, multinational, randomized, double-blind, PBO-controlled trial in RSV-infected lung transplant patients in which the primary endpoint was the effect of ALN-RSV01 on the incidence of new or progressive BOS at Day 180. Secondary endpoints included the impact of ALN-RSV01 on symptom scores, antiviral activity and safety. RSV positive subjects were randomized (1:1) to receive either aerosolized ALN-RSV01 or PBO for 5 days, alongside the hospital's standard-of-care. Subject stratification to treatment arms was based on two binary factors: 1) time from

symptom onset to treatment start and; 2) pre-infection BOS grade. Of the 3,985 subjects prescreened at 33 centers, 218 were RSV positive, of which 87 were randomized. Enrollment is completed and subjects are now in the follow-up phase. Final study results will be presented at this meeting.