ENANTA Pharmaceuticals

From Chemistry to Cures

EDP 938-101 Phase 2a Study: Human Challenge Study

Topline Results

Conference Call and Webcast
June 14, 2019

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This presentation contains forward-looking statements concerning our RSV program, as well as our plans, objectives and expectations for EDP-938 and its development. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "predict," "potential," "positioned," "seek," "should," "target," "will," "would," and other similar expressions that are predictions of or indicate future events and future trends, as well as other comparable terminology. All are forward-looking statements based on our management's current expectations, estimates, forecasts and projections about our business and the industry in which we operate and our management's beliefs and assumptions. These forward-looking statements are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. These risks and uncertainties include: the development risks of early stage development efforts in the disease areas in Enanta's research and development pipeline, such as RSV; the impact of development, regulatory and marketing efforts of others with respect to competitive treatments for RSV; Enanta's limited clinical development experience. As a result, any or all of our forward-looking statements in this presentation may turn out to be inaccurate.

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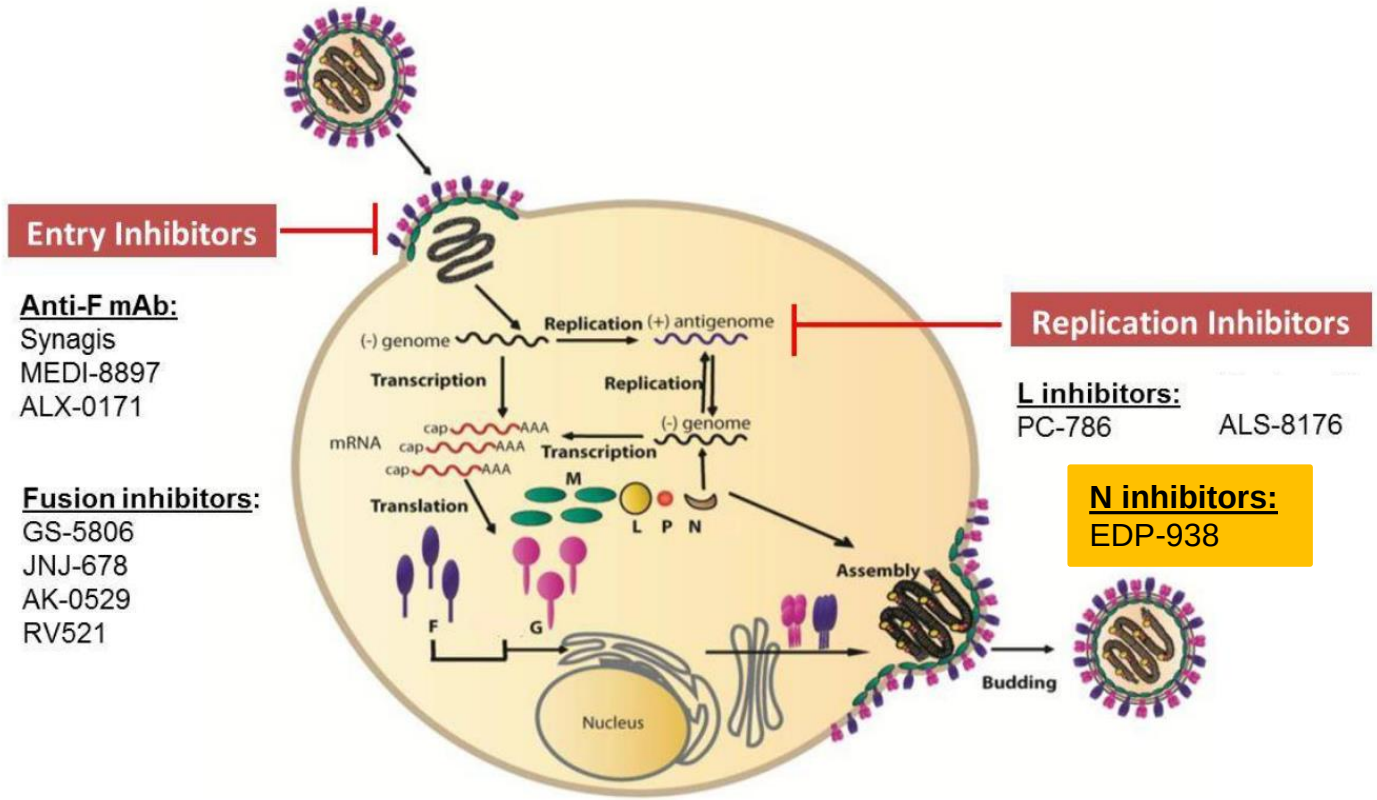
RSV Infection: An Unmet Medical Need

- Respiratory Syncytial Virus (RSV) represents an important global health challenge with significant morbidity and mortality in infants, elderly, and immunocompromised populations
- No approved vaccine to treat or prevent RSV mediated disease is available
  - The only approved antiviral therapy for RSV is ribavirin, but rarely used due to its unfavorable toxicity, its poor antiviral effect, and its controversial and limited efficacy [1, 2]
  - Existing prevention strategies rely on monoclonal antibodies which are only partially effective and which are administered to only a small fraction of the at-risk population [3]
- An effective therapy for RSV infection represents a major unmet medical need

Kimpen (1997), De Vincenzo (2000), Impact-RSV Study Group (1998)

RSV Life Cycle and Targets

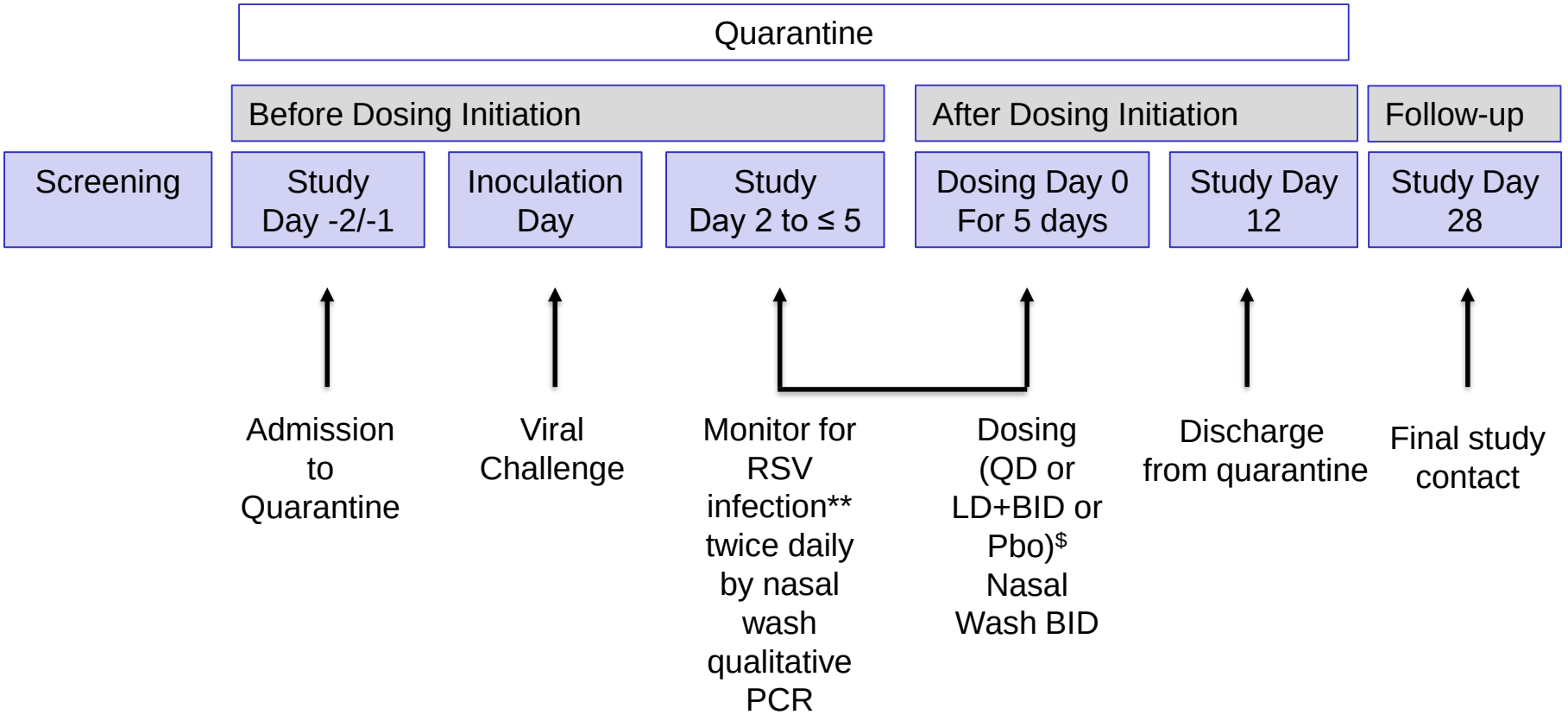


Source: Modified from Najjar et al. Viruses (2014), 6(8):3019-3054
Heylen et al. Biochem Pharmacology (2017), 127:1-12

EDP-938
A Novel, Potent RSV N-Protein Inhibitor

- Active *in vitro* against RSV-A and -B clinical isolates [4-8]
- Demonstrated excellent *in vivo* efficacy, reducing viral load by >4-log in a pre-clinical primate RSV infection model [4-8]
- Phase 1 complete in healthy subjects [9]
  - Safe and well-tolerated over a broad range of single doses up to 800 mg QD and multiple doses up to 600 mg QD or 300 mg BID for 7 days
  - Mean EDP-938 trough exposures were **up to approximately 30x higher than the EC_{90} against RSV-infected human cells**
- Fast Track Designation granted by FDA

Phase 2a Challenge Study (EDP 938-101) Study Design and Procedures



** Dosing (D0) is initiated 12 hours after testing positive for RSV or Day 5 (PM), whichever comes first

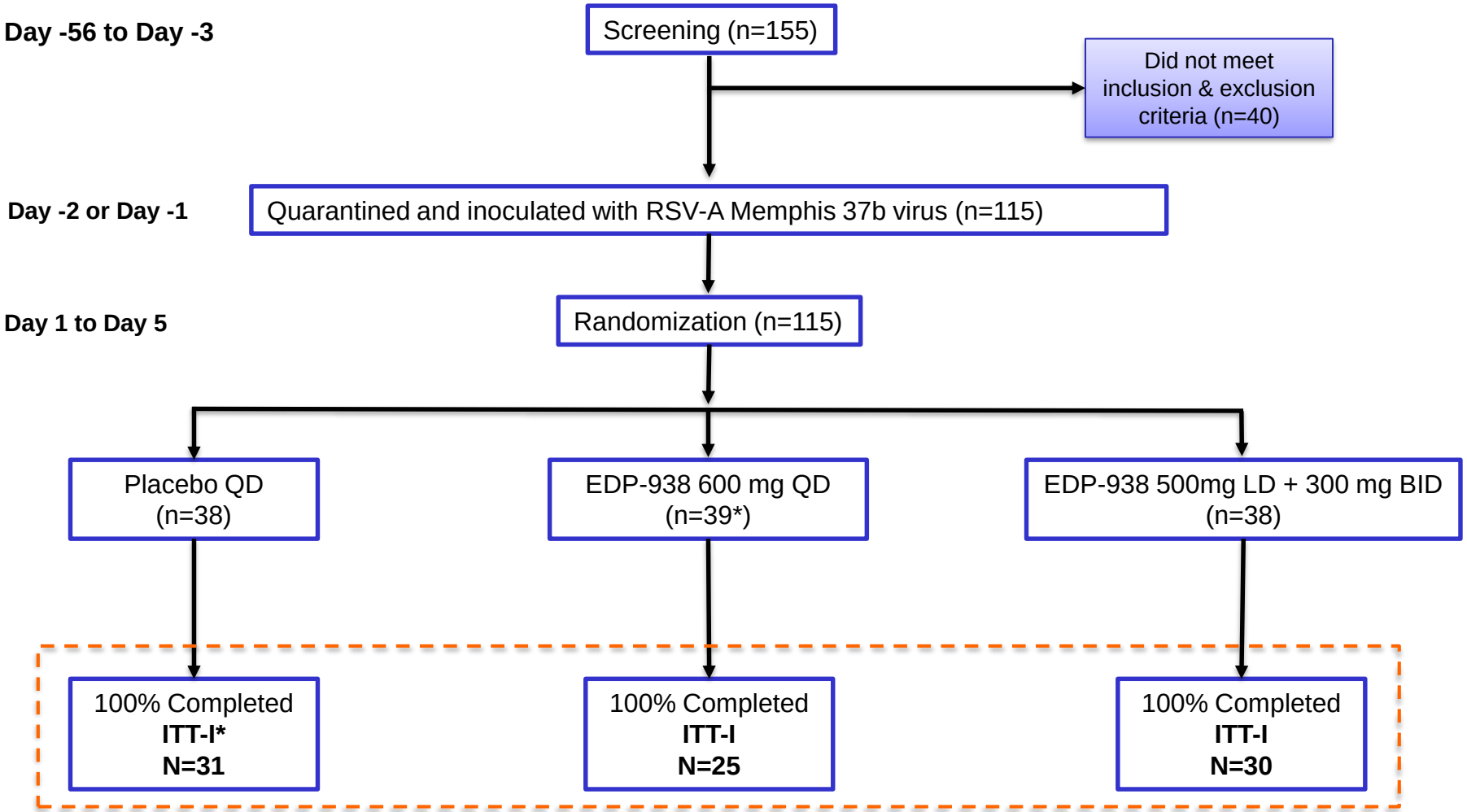
$ EDP-938/placebo is administered as a blinded oral liquid suspension

- EDP-938 500mg loading dose, then 300mg BID over 5 days
- EDP-938 600mg QD Q24h alternating with placebo Q24h x 5 days to maintain the blind
- Placebo for EDP-938 BID x 5 days

EDP 938-101 Study Design
Powered for Both Viral Load and Total Symptom Score (TSS)

- Study designed with an 80% power to detect a 70% reduction with a two-sided alpha=0.05 and assuming an infection rate of 56%
  - RSV Viral Load AUC: Primary Efficacy Endpoint
    - To detect a 70% reduction in RT-qPCR AUC
      - Requirement for 22 inoculated subjects to identify **12 infected per treatment group**
  - Total Symptom Score (TSS): Key Secondary Efficacy Endpoint
    - To detect a 70% reduction in TSS AUC
      - Requirement for 38 inoculated subjects to identify **21 infected per treatment group**

EDP 938-101: Participant Disposition

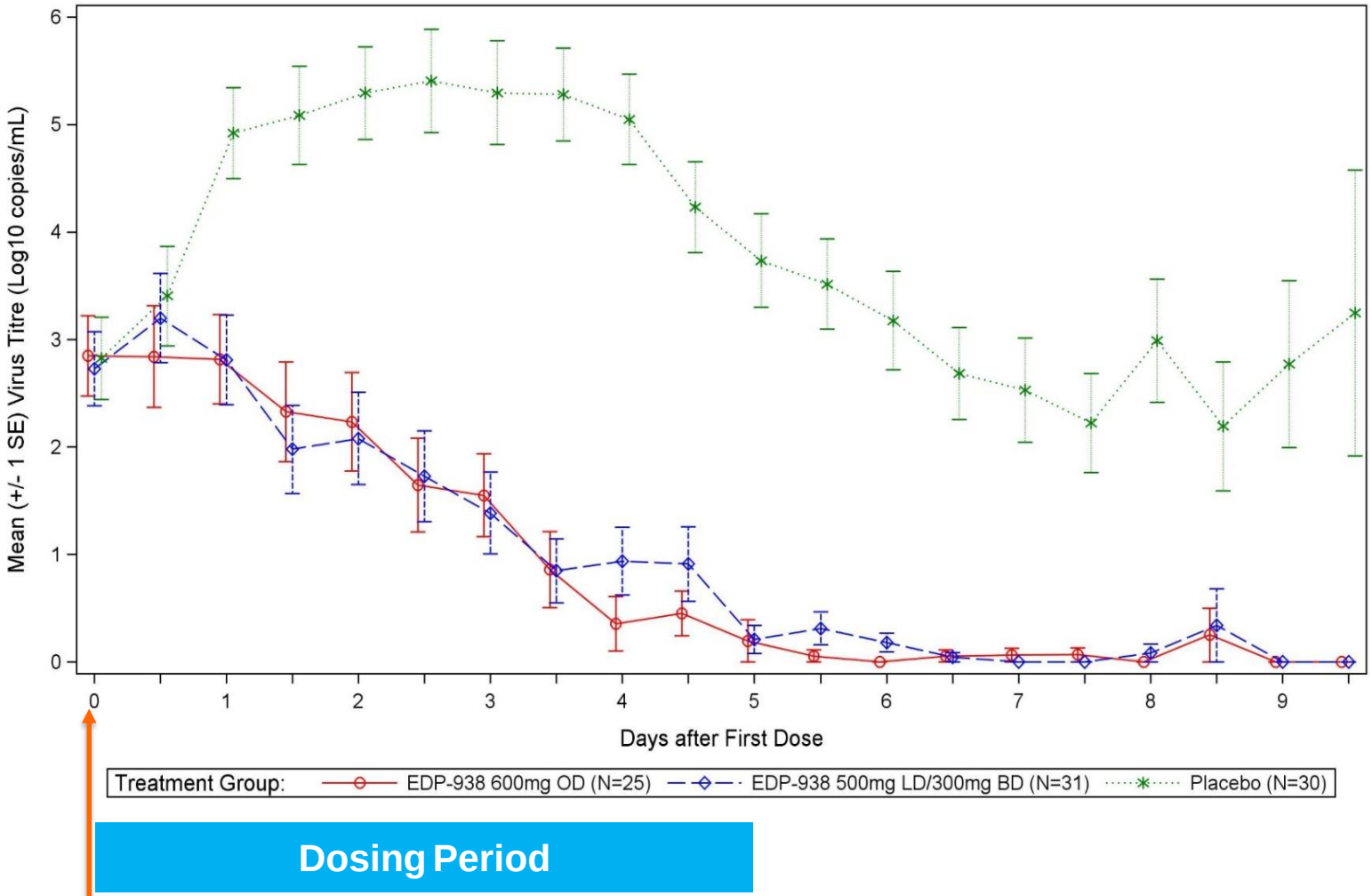


Primary Efficacy analysis, Intent-To-Treat Infected (ITT-I): All randomized subjects receiving challenge virus and ≥1 dose of study drug and with confirmed RSV infection

* One Subject randomized but not dosed. This subject completed the quarantine period

Robust Antiviral Effect

Rapid and Sustained Reduction in Viral Load in Both Active Arms Compared to Placebo



Highly Statistically Significant Reduction in Both EDP-938 Arms Compared to Placebo

- No Statistically Significant Difference Between the Two Dosing Regimens

	EDP-938 600 mg QD	EDP-938 500 mg LD + 300 mg BID	Placebo
N	25	31	30
Viral load AUC mean (SD) (hours x Log_{10} copies/mL)	203.95 (173.50)	217.71 (217.55)	790.15 (408.80)
% Reduction (relative to placebo)	74.43%	71.46%	
Absolute Reduction* (relative to placebo)	-588.08	-564.63	
P-value	**<0.001**	**<0.001**	
Difference between two EDP-938 dosing groups	-23.45		
P-value	0.722		

* Difference in LS Mean

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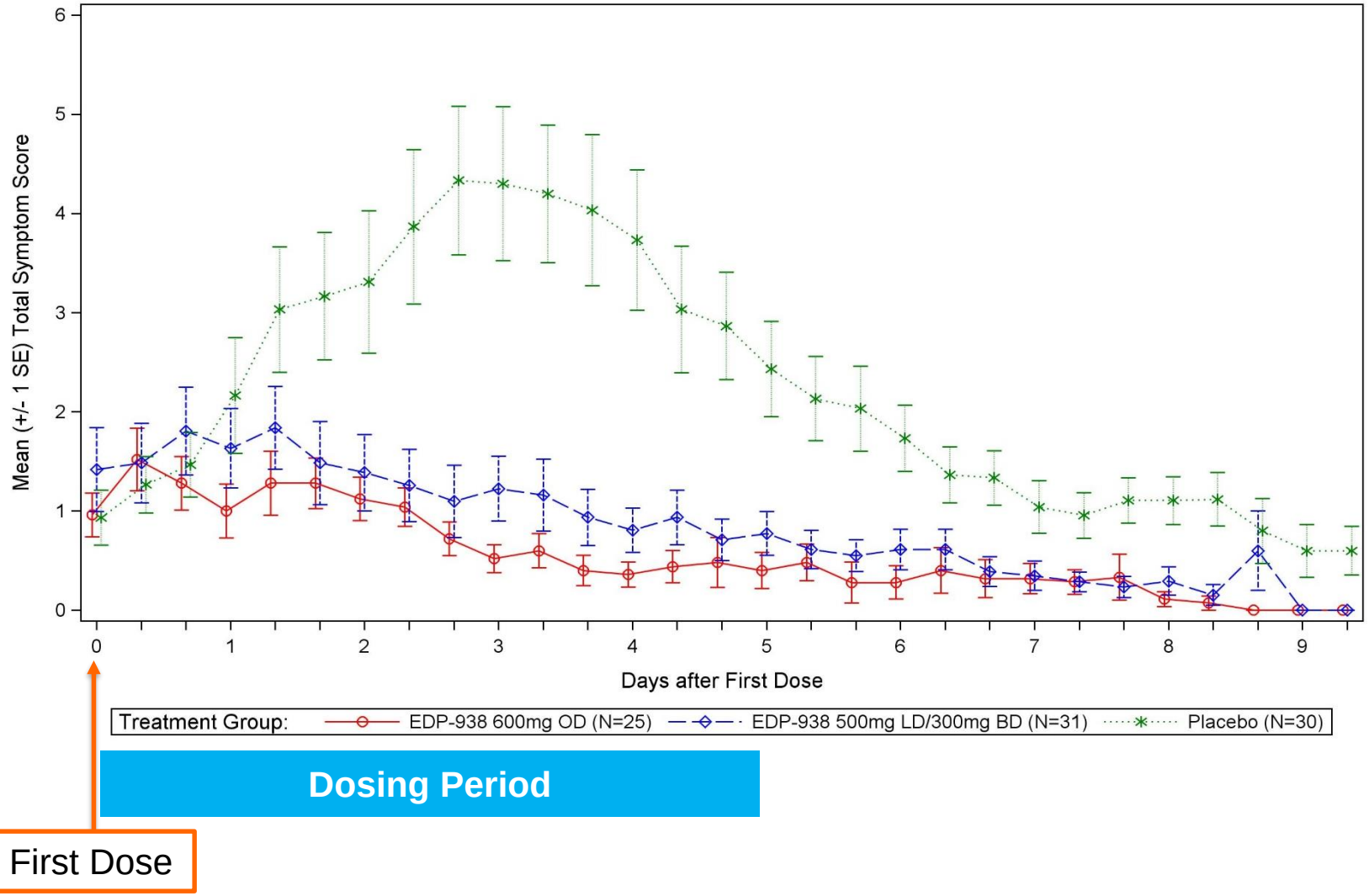
EDP-938 Shows a Rapid and Sustained Attenuation of RSV Symptoms Compared to Placebo



Both EDP-938 Regimens Demonstrated Highly Statistically Significant Attenuation of RSV Symptoms Compared to Placebo

- No Statistically Significant Difference Between the Two Dosing Regimens

	EDP-938 600 mg QD	EDP-938 500 mg LD/300 mg BID	Placebo
N	25	31	30
AUC Total Symptom Score mean (SD) (hours x Score)	124.47 (115.60)	181.75 (248.42)	478.75 (422.29)
% Reduction (relative to placebo)	74.3%	68.2%	
Absolute Reduction* (relative to placebo)	-355.91	-326.64	
P-value	**<0.001**	**<0.001**	
Difference between two EDP-938 dosing groups	-29.27		
P-value	0.700		

* Difference in LS Mean

EDP-938 Was Safe and Well Tolerated in the RSV Human Challenge Study

- EDP-938 demonstrated a favorable safety profile over 5 days of dosing through Day 28 of follow-up
- Comparable to placebo for both QD and BID dosing groups
  - No significant single event or pattern of events was observed compared to placebo
- There were no SAE's and no discontinuations of study drug
- There were no clinically significant laboratory abnormalities in either QD or BID dosing groups compared to placebo

Summary: EDP-938, A Highly Efficacious and Safe RSV N-Inhibitor in the Human Challenge Study

- Primary and Key Secondary Efficacy Endpoints were achieved with high statistical significance at both dose levels (600mg QD and LD 500mg + 300mg BID) after 5 days of dosing
- EDP-938 mean C_{trough} concentrations were maintained at approximately >20-40 fold above the *in vitro* EC_{90} for RSV infected human cells
- EDP-938 regimens were well tolerated with safety profiles that were similar to placebo, a consistent profile that has now been observed in >250 subjects exposed to EDP-938 for up to 7 days

Acknowledgments

- We extend our thanks to the subjects who participated in this study and the hVIVO team and site personnel for their conduct of the study

References

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