

# Catalyst Roundup: A Review of Select Catalysts from Last Quarter's Outlook Report

Each quarter Biomedtracker analysts compile a curated list of key upcoming milestones to feature in the quarterly Outlook Report. Here is an assessment of two catalysts from last quarter's report to review "What Happened?" and "What's Next?"

Drug:  
**Patisiran for TTR-related Hereditary Amyloidosis**  
Alnylam Pharmaceuticals (ALNY)

Catalyst Occurred:  
**Phase III APOLLO - Top-Line Results**  
September 20, 2017

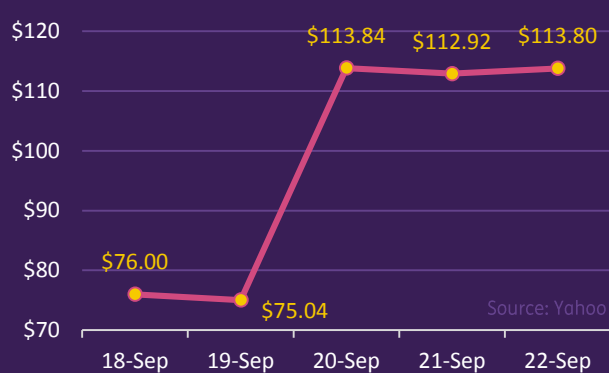
## TRIAL RESULTS

- ☑ Met Primary Endpoint
- ☑ Met All Secondary Endpoints

"The results are particularly reassuring given that the Phase III study included a greater variety of genotypes and more advanced patients than in Phase II."

"Top-line efficacy and safety results reported for APOLLO appeared quite positive, and while officials did not release numerical details on the efficacy findings, their descriptions indicated these were similar to what was expected based on prior data."

## ALNY: 5-DAY STOCK PERFORMANCE



## BIOMEDTRACKER ANALYSES

Change to Likelihood of Approval (LOA): **+15%**

| LOA before results | LOA after results |
|--------------------|-------------------|
| 61%                | 76%               |
| (1% Below Avg.)    | (14% Above Avg.)  |

## NOTABLE UPCOMING CATALYSTS

**NDA & MAA Filings: By end of 2017**

Drug:  
**Translarna for Duchenne Muscular Dystrophy**  
PTC Therapeutics (PTCT)

Catalyst Occurred:  
**FDA Advisory Committee Meeting**  
September 28, 2017

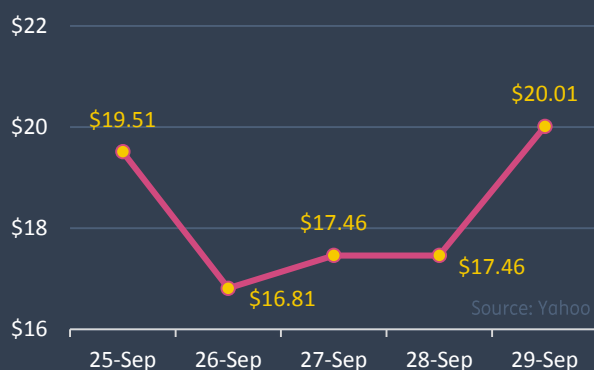
## VOTING RESULTS

**VOTE:** The best interpretation of the information presented today regarding the use of ataluren for the treatment of dystrophinopathies resulting from nonsense mutations in the dystrophin gene is that:

- a. The data suggest that ataluren is not effective  
**0 VOTES**
- b. Although it is possible that ataluren may be effective, the data are inconclusive, and more work would be needed to establish whether ataluren is effective  
**10 VOTES**
- c. The data are sufficient to conclude that ataluren is effective  
**1 VOTE**

"The overwhelming vote that the data are inconclusive came as expected... The drug will not be approved this time around, but the signal for efficacy in the subgroup analysis and rationale for that subgroup are interesting."

## PTCT: 5-DAY STOCK PERFORMANCE



## BIOMEDTRACKER ANALYSES

Change to Likelihood of Approval (LOA): **-5%**

The LOA was adjusted twice, after the brief was released and after the panel voted

| LOA before brief | LOA after brief  | LOA after meeting |
|------------------|------------------|-------------------|
| 82%              | 78%              | 77%               |
| (7% Below Avg.)  | (11% Below Avg.) | (12% Below Avg.)  |

## NOTABLE UPCOMING CATALYSTS

**PDUFA Decision: October 24, 2017**

Interested in further analysis? Visit Biomedtracker to read the latest Outlook Report and discover how you can track real-time drug updates, upcoming catalysts and more.

