

Conversation ID: a24a661c2eb55542903c72391ec09f9b

A meaningful component of that future value is tied to sparsentan. I know much of the focus recently has been on its regulatory pathway. So I'll walk through our update from earlier today.

In November, we followed up with additional positive data from the DUET trial, which was presented in the late-breaking oral session

Further analysis of the safety database from the initial 8-week double-blind treatment period presented at the conference showed that sparsentan was generally safe and well tolerated.

As many of you know, this definition was derived in conjunction with the NEPTUNE Consortium

to complete the analysis and gain agreement with FDA on this particular piece. We are confident we will get there in the very near future.

of FSGS stakeholders to efficiently enroll this trial far more . We have identified more than 100 sites globally with interest in participating in the trial.

but they also felt that they would like to see a longer observation period than the 8 weeks,

They involve known and unknown risks, uncertainties and assumptions that may cause actual results, performance and achievements to differ materially from those expressed or implied by this statement.

To do that, let me first take a step back and set the stage for our interaction with the Food and Drug Administration.

So heading into our end of Phase II meeting with the agency in late January,

So we are pleased to have this clarity on the outline of the trial, as we make further progress on the statistical plans. We'll be in position to give more detail on the specific trial design elements, including patient numbers and observation periods.

We estimate these sites represent more than 1,600 patients who would be potentially eligible

Bill brings a well-proven ability to lead successful research and development organizations

One thing we've learned over the past year is that there are still a significant number of patients diagnosed with cystinuria susceptible to stone formation, and we want to ensure we do our part to educate the treating physicians.

In terms of development support, we are increasingly excited about the added potential to create long-term value with sparsentan. As Steve mentioned, work on assessing additional indications is ongoing, and we look forward to giving more detail in the near future.

we had growth across all of our commercial products during the quarter, which led to the 23% growth over the fourth quarter last year.

We generated value by reaching key milestones that demonstrated sparsentan could represent a significant advancement

which was a controlled observation period in DUET. That was clear. One of the things we are working with them on right now is what is the exact observation period that we want to put into the confirmatory trial.

we built a strong body of evidence supporting the potential benefits of sparsentan. I'm pleased to share that we had a very constructive and insightful interaction with the agency.

The pre-marketing portion of the trial will focus on an interim analysis of proteinuria, which shows a substantial treatment effect,

We look forward to continuing our discussions with the agency to finalize the protocol and initiating the trial later this year.

for entry into the trial. Beyond that, we will continue to work closely with the NEPTUNE Consortium and NephCure international organizations

For Cholbam, the focus for bile acid synthesis disorders will be on supporting the evolving change in the diagnostic paradigm. The team will do this by raising more awareness of our genetic cholestasis panel and increasing its use.

Finally, I'll touch on our business development efforts before turning it over to Laura to run through the financials. BD remains a key piece of our strategy,

are you thinking about maybe -- do you think you would see a change in -- with a level like that or do you think you might have to

They were no issues related to safety that came up in the meeting.

And then we're -- obviously, the second part of that is statistical modeling to see how many patients we need to have reached that time point

We made substantial progress on both the development and commercial fronts in 2016. I'm very proud of our many accomplishments during the year. Most recently, in the fourth quarter, we presented additional positive data

Also notable was the change in the proportion of patients achieving modified partial remission during the open-label period of the study.

We came away from the meeting with a much-needed clarity on how to move sparsentan forward as expeditiously as possible.

It is worth noting that since the DUET results were originally announced and presented last fall, external awareness and excitement around the sparsentan program has grown significantly and the investigator patient and advocacy communities

to ensure we fully involve and leverage the resources of the broader nephrology community.

As you know, our CMO had an unforeseen manufacturing delay, which resulted in us having to move first patient dosed in the trial.

Looking ahead to 2017, we have a number of exciting initiatives that we expect to further our top line growth over 2016. Most notably, we're implementing a small expansion of our sales force, which will allow us to have 2 dedicated teams,

Based on our planned efforts, we expect the use of the panel to double in 2017. For Zellweger spectrum disorders, we will be pushing forward our patient identification efforts

and with further clarity on the sparsentan pathway, we are prioritizing potential transactions to build a sustainable portfolio to serve rare disease patients. I'll now turn the call over to Laura to give you the financial update. Laura?

supported by a commercial portfolio with double-digit year-over-year growth expectations. Combining that with the ability to leverage our strong financial position

The other thing they have asked us to do and which DUET did not do for us

What we do need to do for them is go through those databases, though, and link up

in order to have the state power to be comfortable that we watch statistical significance when we take a look.