CONCERT PHARMACEUTICALS, INC.
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Company Name: Concert Pharmaceuticals Inc. (CNCE) Event: 29th Annual ROTH Conference Date: March 14, 2017 <<Sa'ar Yaniv, Analyst, ROTH Capital Partners>> Hello, everyone thank you for joining us. My name Sa'ar Yaniv, I am the Biotech Analyst here at ROTH. Presenting next is Concert Pharmaceuticals, a biotech Company with market capital of about $380 million. Concert is a biotech company, focused on development of therapeutics for unmet medical needs. The Company's outline is – includes clinical candidates of the treatment of pulmonary, autoimmune, inflammatory and central nervous system diseases with numerous partner products in various phases of development. Presenting for the Company today is Roger Tung, President and CEO. <<Roger Tung, President and Chief Executive Officer>> Thanks very much. I appreciate the introduction and I'd like to start out by thanking the ROTH Health Care team for the very kind offer to come and present today. It is pleasure and I'm very excited to be speaking to you about our developing clinical pipeline including the work that we're doing on CTP-656, about which we had a recent announcement of a deal with Vertex that I'll be talking about. So I would like to note that during the presentation today I will be making forward- looking statements and for details on the meaning and content of those statements please refer to our recent 10-K filed with the Securities and Exchange Commission. I will note also that during the presentation, I'll be talking about our pending transaction with Vertex Pharmaceuticals. Details on that transaction will be filed in the coming couple of weeks in a proxy statement with the SEC, and will be available on their website or you can contact the Investor Relations at Concert Pharmaceuticals for more details. So I'd like to start by noting that we have quite a bit going on at the Company. We've recently announced that we are going to enter into a asset purchase agreement with Vertex Pharmaceuticals on our novel potentiator CTP-656 and that will be an important event for the Concert on it's closure. I'll note that from a patient perspective, we think that this is a great outcome for the compound enabling it to reach patients faster and to reach a broader number of patients in particular, the homozygous F508 deletion patients which a monotherapy would not be able to access. But for shareholders of Concert Pharmaceuticals it's important because it provides us with the fuel to take our next program CTP-543 for the treatment of alopecia areata forward without requiring a dilutive equity raise.

And we project that based on current operational projections, we will have enough cash to bring us into 2021 on closure of the deal based on the upfront cash received. We have quite a bit going on in the Company. We have CTP-543, as I noted, which will be entering into Phase 2 studies for alopecia areata we project this month. And with CTP-656, we are conducting a U.S. Phase 2 study in the gating mutation population, which is a small population within the cystic fibrosis community for which Kalydeco, of which the active ingredient is ivacaftor is currently approved. That study is ongoing and enrolling now. Also under our agreement with Avanir Pharmaceuticals a subsidiary of Otsuka, the Phase 3 studies for AVP-786, which contains deuterium- modified dextromethorphan, that we licensed to them those studies are ongoing and we project the possibility of the completion of those studies and commercialization of the entity within the timeframe of our cash guidance pending positive results. And I'd like to note that in terms of the way that we used deuterium technology, there are really two kind of broad buckets or two prongs, in which we applied the technology. The first of them typified by CTP-656, this is a situation where we used deuterium technology to substantially modify the pharmacokinetic characteristics of the underlying drug while still retaining the pharmacological activity of it, but alter it in a way that it makes it potentially a substantially better drug for the indication, in which it was originally approved. A second way, in which we use the technology is exemplified in CTP-543 and AVP-786 where through deuterium modification, we've differentiated the compound and created a new composition of matter, but our intended use of technology is to take it outside of the previously approved indications and into a potential first-in-class indication. And I'll be talking more about each of these cases during the course of the presentation. So CTP-656 is an agent that we believe could be a best-in-class potentiator for the treatment of cystic fibrosis. So a lot of work that's ongoing in CF at present and some of the most exciting work is that, which affects the underlying mechanism of the disease which are defects in the CFTR protein. A lot of different mutations can affect that protein, but what is common is that the so- called potentiator class of compounds generally improves the flow of ions through the channel regardless of the mutational status as long as the protein has any activity. So in that way we believe that it will be combined with other mechanisms and form the backbone of therapy going forward. We created CTP-656 to address what we saw as potential shortcomings in Kalydeco, which is the only approved and standard of care treatment for gating mutations. And is also used in a combination drug called Orkambi which is approved for the treatment of the larger – the largest cystic fibrosis population with homozygous delta F508 mutations. And in particular CTP-656 produces substantially greater exposure in a much longer half life, then does ivacaftor or non-deuterated CTP-656, enabling potentially a once-daily treatment and also a more compact regimen which will be, we believe more patient-

friendly and easier to adhere to than drugs containing ivacaftor itself. And it really is on the basis of the data that we have collected with the Phase 1 studies, of CTP-656 that we and Vertex entered into the agreement that was announced very recently. So a little bit more background about the detail. As I noted the potential value of the deal is $250 million. And very importantly for us and for shareholders, the bulk of that amount, $160 million will be transferred on closing of the deal. There will be $90 million in potential milestones both of them pre-commercial, $15 million will be based on approval in the U.S. and $40 million will be based on Vertex obtaining a pricing and reimbursement agreement in one of three European countries, the first to be either Great Britain, Germany, or France. So we think that this is a, as I noted, a great outcome for the drug in the sense that Vertex has got the broadest pipeline of CF modulators that are in the business, their ability to combine their pipeline with CTP-656 to create new combination medicines. It is important I should note actually that the milestones are based on approval of a combination medicine not just a monotherapy. But we believe that Vertex is highly incented. And we'll be working hard to produce new medicines based on combinations of their pipelines with CTP-656 in a more convenient form. Now I'll note that the deal is pending and closure of it will be dependent on shareholder approval, as well as typical types of agreements, such as HSR approval. So we anticipate that that will happen later this year. And upon closing of the deal based on the $160 million up front, we project that this will enable us to move our CTP-543 alopecia areata program through Phase 2 into pivotal registration trials. We anticipate that we'll be able to strengthen our pipeline by bringing new proprietary compounds into it. And we also think that there is the potential to have AVP-786 proceed through its Phase 3 program and possibly into commercialization to allow Concert to begin realizing royalties on that product. And I'll talk about that in subsequent slide. Now we're continuing to conduct the Phase 2 program that we started last December on CTP-656. Just as a reminder this is a study that's carried out in the rare gating mutation population of cystic fibrosis patients, notably those with the G551D mutation. We're projecting an enrollment of 30 patients to 40 patients with three active arms of CTP-656 of 20 milligrams, 100 milligrams and 150 milligrams, versus placebo. And there will also be an open-label Kalydeco arm, or there is an open-label Kalydeco arm that is conducted in parallel with the blinded arms. The primary endpoint on the study is sweat chloride and the study is powered for it even with these relatively small numbers. FEV1 will be an important endpoint and we also will be measuring it throughout the study. We are continuing it as planned and actively enrolling it. We continue to project that top line data will be available in Q4 of 2017. This of course is pending the transfer of the program to Vertex through the asset purchase agreement.

I'd like to then move on to CTP-543, which we're very excited about. This is a compound that is potentially we believe the first oral treatment for alopecia areata that can reach approval. So this has the opportunity to address an unmet need for which there are no currently approved therapies, but there is we believe a strong pent up need and demand. We're targeting the more severe form of the disease. What we're referring to as moderate-to-severe alopecia areata, where 50% or more of scalp hair is lost. And this is a form of disease which is typically not a remitting form, so these patients will have that indication for years and often it's lifelong. So alopecia areata just by way of background is a – it's a fairly common autoimmune disease that involves attack of the hair follicles by cytotoxic T cells resulting in inflammation and loss of the growing hair and results in patchy loss of hair, or sometimes complete baldness and in the most severe forms alopecia universalis, loss of all body hair. We are working with the deuterium-modified version of ruxolitinib, which is a selective JAK1 and JAK2 or Janus kinase 1, Janus kinase 2 inhibitor. The approved form of ruxolitinib, known as Jakafi in the United States is approved for the treatment of certain blood disorders, including myelofibrosis and central thrombocytopenia. The exciting thing to us about this program is that we have what we believe is a pretty compelling clinical proof-of-concept that ruxolitinib will be effective in this disease state. We do not believe that ruxolitinib itself will be taken as an oral medication into this indication for a number of reasons, largely revolving around price point. We have taken now the CTP-543 program through its Phase 1 single and multiple ascending doses studies and are poised to enter into Phase 2 later this month. Now our understanding is that there's upwards of 0.5, million maybe up to 650 patients in the U.S. at any given time who are affected by alopecia areata. This, as I noted in it's more severe forms, is a chronic condition and we do believe that the treatment of the disease with JAK inhibitors will be a chronic treatment, we see this as a significant and important disease state. We believe that the FDA agrees with that which again leads to our segment in being able to bring forward a new medicine to treat it. For many of the patients, who have alopecia areata it is a really devastating disease that can have severe effects psychologically and is also often co-morbid with other autoimmune diseases. And this is one of the relatively few areas, eight areas that the FDA has selected for its R016, 2017 patient-focused drug development initiative meetings which are meetings whereby FDA gathers patients from the community who experience a different disease state. So that they can get informed about the effect of the indication on the patients and therefore understand the risk benefit tradeoff, which is appropriate for the disease state. And we think that the fact that they're taking the time and the effort to have this meeting is an indication as they've stated to us that they view this as a serious disease and one that really deserves focus on developing a new treatment. Now as I mentioned there is what we think is a really very important clinical proof-of-concept for the Janus kinase-1/Janus kinase-2 approach for treating alopecia areata. There

was a study that was conducted by Columbia University the primary investigator was Julian Mackay-Wiggan on this study and that was published last year. And her group demonstrated that in a cohort of a dozen patients who are treated with ruxolitinib for alopecia areata that out of – that 12 patients nine responders were noted. Responders being defined in that case as those that had a 50% or greater remission of alopecia areata over the course of three to six months, of the nine responders each of them are shown in before and after photo along this set. And as you can see the effects of the drug in many of the patients was profound, a really pretty unprecedented results. And of the individuals who had regrowth of hair, the average regrowth was 90% or greater. So something that we think is speaks volumes to the efficacy of the mechanism. Importantly, the treatment was well-tolerated there were no severe adverse events associated with it, there were – effects that were associated with the mechanism disease including in one case a mild red blood cell anemia, which resulted on dose reduction and did not recur on re-challenge with the drug. As I noted, we have now conducted our Phase 1 studies with the program enabling us to enter into Phase 2, we see that exposure as we had hoped and predicted with CTP-543 in healthy volunteers is greater than would be expected for exposure of a similar amount of ruxolitinib. And the drug is well behaved and we think in a good position to move forward there were no serious adverse events reported in our Phase 1s, no drop-offs and no dose reductions during the study. Importantly, we followed several of the pharmacodynamic markers of Janus kinase inhibition we noted a dose dependent reduction in the STAT3 phosphorylation which is – which mediates more of a blood cell population effect. But we saw a very strong effect in interferon-gamma-mediated STAT1 phosphorylation and this is a particular importance because interferon-gamma is postulated to be one of the key drivers for alopecia areata. So seeing a very powerful effect on the gamma or interferon-gamma, biochemical pathways is important and something we're very pleased about. We are poised to enter the Phase 2a study and this will be a double-blind, placebo- controlled dose ranging study in multiple centers looking at four different active doses of CTP-543 each twice-daily for 8, 12 and 16 milligrams versus placebo, about a 100 patients total. We expect fast enrollment for this and are hoping to have top line data reported out by the end of this year based on 24 weeks of active treatment. This study is a one-year study and the reason that we're continuing it out beyond the primary endpoint is to collect more chronic efficacy data as well as to better understand the safety benefits or the safety efficacy profile of the drug at different doses. And I'd like to move then very briefly to our last clinical program being conducted as I noted by Avanir Pharmaceuticals and entirely paid for by them. AVP-786 is a potential first-in-class treatment for agitation and aggression symptoms in patients with dementia secondary to Alzheimer's disease. Again a situation where there are no approved treatments. This is a very large unmet medical need and one where we think that's having an approved label drug could be extremely beneficial to that patient population and a very

important economic benefit to Otsuka and Avanir and of course to us based on the royalties and milestone stream that we have potentially available to us. Avanir currently has two Phase 3 studies ongoing. They are looking for the completion of those currently for clinicaltrials.gov in Q3 of 2018. We have potential for milestones as I noted of $170 million of which only $8 million have been attained thus far. So most of them are sales based and there are some regulatory milestones that we have available to us. And mid single to low double-digit royalties are based on sales of the drug on a tiered basis country by country, which would be potentially very meaningful to us given our cash situation. We yet exited last year with $96 million, slightly over $96 million in cash. Pending the closure of the Vertex transaction, we expect to have over $250 million pro forma available to us, which certainly changes the completion of our ability to act without having any further dilution of the shares. And with that, I'd like to note that we have a good number of upcoming milestones during the year. CTP-656, closing of the asset purchase agreement and reporting the top line efficacy data, again, pending the closing of that transaction. CTP-543, we've checked off a couple of the boxes already and initiation of Phase 2a should start shortly with we hope a report of the Phase 2a top line results at the end of the year. With that, I'd like to stop and then I'm happy to answer any questions. Q&A <Q – Sa'ar Yaniv>: Thank you so much, Roger. Any questions from the audience? So, I'll start, and I wanted to know if you can talk a bit about the Vertex because it seems a little uncharacteristic for biotech deals, which usually have a smaller upfront and larger milestones and royalties attached to it. Can you talk a little bit about the reason you started the deal that way? <A – Roger Tung>: Sure, I think it's balanced or different interests. And as you know in biotech deals, you can put theoretical diligence clauses in, but they're very hard to actually enforce. And we believe in that Vertex has a very strong interest in developing CTP-656, but we have no way of exerting leverage over that and it's particularly – it's a deal where they own an existing drug ivacaftor, so we wanted to make sure that we were able to access a large amount of the value for it upfront. The way that this is structured, we believe in sense Vertex to develop CTP-656, because they have a relatively modest series of payments to us based on pre-commercial milestones. And on commercialization they won't owe for royalties, but the value that we're getting from this deal are certainly the $160 million upfront and potentially the $250 million overall we think is a tremendous return on our investment, it gives us the ability to bring our pipeline further down and really furthers our interests as an organization that wants to

make sure that both CTP-656 gets developed as a medicine, but also wants to develop our own pipeline further. <Q – Sa'ar Yaniv>: Great, thank you. And now that you bring up the pipeline question, you have quite a diversified pipeline and you've numerous product developments in numerous therapeutic areas. So do you expect to – when you look in the CTP-543 for alopecia, do you expect to take that to the clinic by yourself – sorry, all the way to approval by yourself or partner like the other drug development? <A – Roger Tung>: Well, our baseline view right now is that with CTP-543 one of the reasons that we really liked the indication as well as the medicine, but the indication is that there's a tremendous amount of unmet need. We think FDA is really incented to move compounds along if a sponsor can demonstrate a good balance of safety and efficacy. We think that the population is one that's highly concentrated in high prescribe or dermatologist. So it is lends itself to something where a tractable sales force for a small company can actually detail the medicine. So this isn't the situation where we think that it's really pretty well suited for a company like ours to take forward all the way to commercialization and really grow our company into that. It also has the potential, because there are the mechanism has a lot of possible other uses you can have something of a pipeline in a product. So there's a lot of things that we like about that that particular molecule. So our focus right now is on closing the CTP-656 deal that really is kind of where all of our effort is going to be. And having accomplished that, then we'll have a high class problem figuring out where else we want to expend our resources. <<Sa'ar Yaniv, Analyst, ROTH Capital Partners>> Great. Thank you so much.