

**CATALYST PHARMACEUTICALS, INC.**  
**Commercial Launch of Firdapse**  
**Transcript of Conference Call**  
**December-13-2018**

Operator: Greetings and welcome to the Catalyst Pharmaceuticals conference call to discuss the commercial launch of Firdapse. At this time, all participants are in a listen-only mode. A question and answer session will follow the formal presentation. If anyone should require operator assistance during the conference, please press star, zero on your telephone keypad. As a reminder, this conference is being recorded.

It is now my pleasure to introduce your host Ali Grande, CFO. Please go ahead.

Alicia Grande: Thank you, Operator. Good morning everyone and thank you for joining us today to discuss the commercial launch of Firdapse. Before we begin, I would like to remind you that in the following comments and the question and answer session will include forward-looking statements based on current expectations. Such statements represent our judgment as of today and may involve risks and uncertainties. Please refer to our filings with the SEC which are available from the SEC or on our website for information concerning the risk factors that could affect our company and the launch of Firdapse.

Joining me on today's call are Pat McEnany, our Chairman and CEO; Dan Brennan, our Chief Commercial Officer; Dr. Steve Miller, our Chief Operating Officer and CSO; Dr. Gary Ingenito, our Chief Medical Officer; and Brian Elsbernd, our Senior Vice President of Legal and Compliance.

As we always do on our calls, we will take questions from analysts after our prepared remarks. I will now turn the call over to Pat.

Patrick McEnany: Thank you, Ali, and good morning and thanks to everyone for joining us on today's call. As you can imagine, this has been a very exciting couple of weeks for the Catalyst team, and we were thrilled to have ended November with the FDA approval for Firdapse. This represents the culmination of many years of hard work and investment. After completing the comprehensive clinical and nonclinical development programs that were required for us to obtain FDA approval of Firdapse as a treatment for LEMS, we are gratified to finally be able to deliver a much-needed evidence-based therapy to all LEMS patients in the U.S.

For nearly 40 years, LEMS patients have relied on investigational drugs with very limited access or off-label, unapproved therapies with no proven safety or efficacy to treat this debilitating disease. With the approval of Firdapse, we believe that there is new hope for the lives of these patients to change for the better.

Firdapse is the first and only FDA approved evidence-based therapy that addresses the dysfunction in the neuromuscular junction, affecting muscle contraction and strength of patients suffering from LEMS.

The most common symptoms of LEMS are proximal muscle weakness and abnormally rapid fatigue. Currently, more than half the people with LEMS receive an initial misdiagnosis and they often wait years for a proper confirmed LEMS diagnosis. Symptoms can be life-threatening when the weakness involves respiratory muscles. Approximately 50% of LEMS patients have an underlying malignancy, typically small cell lung cancer.

Firdapse, or amifampridine, has been approved as a new molecular entity, meaning that the molecule has never previously been approved by the FDA for any indication. That also means that Firdapse is not a repurposed drug or one that has previously been approved for another indication.

Catalyst and BioMarin have spent more than nine years developing Firdapse. Combined, we have conducted more than 70 clinical and nonclinical studies, including two positive Phase III studies at a cost of tens of millions of dollars.

By treating LEMS patients with Firdapse, doctors will be able to help these patients to improve their ability to conduct the simple daily activities that many of us take for granted. LEMS patients have a limited ability to care for themselves and often cannot hold a job requiring standing, moving or walking and it is difficult for them to participate in everyday activities such as drying their hair, cooking dinner, going up and down stairs or walking down a long hallway. We believe that our efforts to study this medicine and bring it through the FDA review and approval process will make a meaningful difference in the lives of LEMS patients and the lives of their caregivers and loved ones.

Our mission does not end with the approval of Firdapse for LEMS. As you know, we are conducting—continuing to evaluate Firdapse in clinical trials for other rare neuromuscular diseases such as congenital myasthenic syndromes, MuSK antibody positive myasthenia gravis, and spinal muscular atrophy type 3. Additionally, we are in the early stages of our work to develop a longer-acting formulation of Firdapse that would require once or twice per day dosing versus currently three to four times per day. We hope that in addition to being a more patient-friendly dosage form, leading to better compliance, it would also ensure an optimum level of efficacy throughout the day as well as throughout the night.

I would now like to address the commercial opportunity that Firdapse represents. We previously reported from the literature in our third-party initiated research studies that there are approximately 3,000 adult LEMS patients in the U.S. However, based on precedence in the orphan space, we have also noted on many occasions that there is a high degree of inherent variability with these numbers when using top-down prevalence estimates for rare diseases. This is due to the small number of patients and the even smaller sample size used in prevalence

studies. Please note that this is not unusual, especially with ultra-orphan diseases for which there is no approved therapies.

Recently, our Commercial Analytics Team initiated a study with a zero-based bottom-up approach of the overall LEMS prevalence, as well as today's addressable diagnosed market to better quantify estimates. This process involved a thorough review of published epidemiology studies, healthcare claims transactions as well as hospital discharge data and other triangulation methodologies. This process has led us to continue to believe that there are approximately 3,000 patients in the U.S. who suffer from LEMS of which approximately half, or 1,500, have been definitively identified in claims data over the past two years. This estimate of the diagnosed patient population compares well with the epidemiology studies in the literature, and we have defined the approximate 1,500 patients as our immediate addressable market. The other half have low visibility in the claims data or are misdiagnosed or undiagnosed. We are also planning steps to help the neurology community identify the other half for further diagnosis and treatment.

As you probably know, another company has been providing a different form of 3,4 diaminopyridine to patients at no cost for a number of years as an investigational drug under INDs for patients with LEMS and CMS. We at Catalyst commend them for this effort. At the same time, Catalyst has also been providing Firdapse for several years at no cost to patients as part of our Expanded Access Program. We believe that between the two programs approximately 10% of all LEMS patients in the patient community currently are on therapy. The reason for this low percentage is that until now there has not been an FDA approved drug that provided the necessary ease of use, pharmacovigilance, FDA approved dosing administration and safety information. Physicians have expressed great concern with the difficult, cumbersome process required to file and maintain their own investigator INDs for compassionate use. Also, many patients were required to travel hundreds of miles to have access to a participating physician for diagnosis and the receipt of drug. This presented a challenging situation for many physicians and patients who were either not aware of or unable to access this potentially beneficial treatment.

Our rationale for pursuing FDA approval for Firdapse was to ensure that all, all patients would have access to an evidence-based treatment with safety and efficacy properly studied and characterized, helping more than just the 10% of patients who are currently in an early access investigational program.

With the FDA approval of Firdapse, any physician will soon be able to write a prescription for their LEMS patients diagnosed and in a few days have Firdapse delivered to his or her door from a rare disease experienced specialty pharmacy. So no matter where they live in the U.S. they will have the drug delivered to their doorstep.

We recognize that the price for medicines and the insurance coverage barriers of significant concern to patients, physicians, healthcare providers, payers and policy makers. We have a

comprehensive array of services under the program we are calling Catalyst Pathways, which we designed to be a single source for personalized treatment support, education and guidance, including a comprehensive patient insurance navigation and financial assistance program designed so that a patient is out of pocket a minimal amount of money. We will be further describing the scope and breadth of our Catalyst Pathways program in detail in a few moments.

Over the past six months we have completed rigorous pricing and access study with a highly regarded and recognized consulting firm. This firm has specific, relevant experience in pricing multiple orphan drug products with commercial success. As part of this process, our Market Access Team met with most of the top commercial and government payers in the U.S. to describe LEMS and introduce our upcoming approval of Firdapse. In addition, we have held focus groups with general neurologists and a neuromuscular specialist in order to obtain feedback about potential plans to enable affordable market access to all patients. This information and other factors were considered in our establishment of the annual list cost of therapy.

Based on the results of this extensive work, we have set the annual list price or wholesale acquisition cost of Firdapse for a typical patient based on average dosing in our clinical trials and the Expanded Access Program at \$375,000 before discounts and rebates. At this price level, it gives the experienced patients adherence and compliance rates, our pricing strategy reflects our focus on ensuring broad and sustainable coverage from both private and public payers and assistance for patients in need. We have also factored in continued clinical investment in the product to evaluate it for other neuromuscular diseases as well as future development of a new sustained-release formulation.

Catalyst has not spared any effort to be prepared for a successful launch of Firdapse, introducing a transformative milestone in the lives of patients in the U.S. who are suffering from LEMS and giving us an expanded patient population access to a first-in-class and proven therapy.

At this time I'd like to invite Dan Brennan, our Chief Commercial Officer, to provide greater detail of our launch plans. Dan?

Dan Brennan: Thank you, Pat, and good morning to everyone on today's call. I've been excited to be part of this company and team since the early summer. I believe at the time I was Employee number 25. One of the very first elements of ensuring the successful commercialization of Firdapse was to assemble a truly world class Commercial Team of passionate, experienced and patient-focused healthcare industry veterans and partners. These individuals came to Catalyst from leadership roles at rare disease biopharmaceutical companies, non-profit patient organizations, rare disease patient support service and specialty pharmacy organizations. Many bring specific expertise in successfully launching orphan neurology products. This is a tight group of highly experienced team members committed to making a difference in the lives of all LEMS patients and their family members.

One of the first areas where we were able to leverage our rare disease experience was in our discussions with payers to provide a disease state overview and the value proposition that Firdapse offers their LEMS patients. Our market research shows that most LEMS patients have insurance coverage and that breakdown is approximately 30% with commercial plans versus 40% Medicare, 15% Medicaid, 10% dually eligible for both Medicare and Medicaid, and about 5% with other federal programs or cash pay. We have had several national account managers with rare orphan drug experience targeting and speaking with over 70 key payer accounts that cover over 80% of the U.S. insured population.

In these discussions and extensive research with payers, we were able to describe the difficulty that people suffering with LEMS experience in their daily lives and the overall medical and personal burden placed on the healthcare system without having any approved treatments. Not surprisingly, the payers were not immediately aware of LEMS given the small number of patients, but after reviewing the disease and the impact it had on their patients, they realized that there is a clear, unmet need for treatment options with established safety and efficacy. They also appreciated that this is a rare condition, currently affecting approximately 3,000 patients and fewer diagnosed patients.

Finally, they understand that as an orphan disease, the exclusivity and orphan pricing window is seven years before generic price and competition is available.

As our team continues to further discussions now post approval, we expect our clinical study results with sustained improvements and a relatively straightforward safety profile will establish the value Firdapse can bring to these LEMS patients who may be rare but are certainly in need of an effective therapy.

Now, by straightforward safety profile, I mean that due to the risk of seizures and allergic reactions, Firdapse is contraindicated in patients with a history of seizures or allergic reactions to any aminopyridine product. The most common adverse reactions that occurred in more than 10% of trial subjects are paresthesia, which is a tingling or prickling sensation, upper respiratory tract infection, abdominal pain, nausea, diarrhea, headache, elevated liver enzymes, back pain, hypertension or muscle spasms.

Based on the direct feedback we've had from payers, we expect the majority of them to cover and reimburse Firdapse with stipulations that are typical for other rare disease covered medications. Our clinical trial results along with the programs we are putting in place will provide payers with the confidence they need in order to make a positive coverage determination and ensure the drug is being utilized and efficacious in LEMS patients. There are clear diagnostic antibody and/or electromyography tests that can positively confirm a LEMS diagnosis, and finally, with Firdapse's rapid onset of action, a therapeutic response will be evident to both patients and physicians quickly so payers can be confident that continued use

and reimbursement for the drug will occur only with a positive effect. I will describe the program we have related to that later in a few moments.

All of those health payers we talked to indicated that they would provide coverage for their LEMS patients at a price that Pat mentioned previously.

So, now to our commercial planning. Our Firdapse launch readiness planning has five prioritized key areas: first, the development of Catalyst Pathways, our concierge-like patient support services program; second, commercial availability and transition planning for patients on Firdapse study drug or investigational 3,4-DAP without having a lapse in therapy. Third, patient identification and awareness of Firdapse for LEMS diagnosed patients who have not had access to Firdapse or 3,4-DAP. Four, rapid comprehensive reimbursement approval with the majority of payers; and five, continued listening to the LEMS patient community and adjusting to their needs which has helped us in each of the previously mentioned areas.

I'd like to go more in depth with each of these five priority areas which we call critical success factors in helping us accomplish a successful launch. I'll start with Catalyst Pathways.

Our Catalyst Pathways program has been designed to provide all people diagnosed with LEMS and their physicians a single-source or personalized treatment support, disease and product education, navigation through insurance coverage on their prescription drug benefit, and information and access to financial assistance programs. Specific details on the program include documentation of a confirmed diagnosis of LEMS; one-on-one educational support and communication with patients and providers through the initial dosing and titration regimen to help efficiently achieve their optimal therapeutic dose; comprehensive patient insurance navigation designed to help providers understand what insurers are requiring for coverage; conducting benefits investigations in communications with insurers; and, support for the appeals process for initially denied claims.

An array of financial assistance programs are available to address patient co-pays and deductibles to a nominal affordable amount.

For patients with commercial coverage, a co-pay assistance program designed to keep out-of-pocket costs to \$10 or less per month will be available. For patients that Catalyst cannot directly support such as government insured patients, Catalyst is providing donations to independent qualified and reputable third-party foundations who financially support the care of LEMS patients. While our donations do not necessarily cover our product, Catalyst is committed to helping the LEMS community.

In addition, Catalyst Pathways provides door-to-door product shipment, scheduling and delivery of medication via our exclusive rare disease experienced specialty pharmacy. Catalyst Pathways is also the gateway for our free bridge medication for patients during transitioning from our EAP study or those on investigational 3,4-DAP who are waiting for a coverage

determination. It is also the access point for our Patient Assistance Program or PAP program which provides longer-term charitable medication for those who are uninsured or unable to obtain coverage despite insurance.

We're determined to achieve high levels of satisfaction from patients and physicians who interact with this Catalyst Pathways program and we'll regularly survey and measure levels of satisfaction from those who come into contact with it.

As of December 1, we are fully staffed and these individuals are already answering calls and providing available information. I know they are happy that today has arrived so that they can start communicating more in depth about the process for prescribing and obtaining medication, actual dates that Firdapse will be available for shipment, and they can now begin communicating with insurers about specific patients and their physicians who we know have already contacted us wanting to understand how and when they can receive this medication.

Our second priority, knowing the Catalyst Pathways program is up and running, is to have commercial availability of product for patients who are transitioning from Firdapse or investigational 3,4-DAP. We aim to transition them to Firdapse without a break in their medicine supply. We know that this is a concern for patients and we have allocated our initial supply for them by January 15th. Beginning today, patients and their physicians can start the process and fill out the enrollment, consent and prescription form off of our Catalyst Pathways website which is located at [www.yourcatalystpathways.com](http://www.yourcatalystpathways.com).

Once Catalyst Pathways receives an enrollment form fully completed by physician and patient, our Catalyst Pathways personnel will help facilitate communications with the payer to determine coverage, obtain reimbursement and provide information on our co-pay support programs or financial assistance programs in order to quickly obtain the medication. We will also have free bridge medication supply available to them starting January 15th to help during the early days of coverage determination.

Our third priority is to ensure commercial readiness for all other LEMS diagnosed patients, and to focus on patient identification and physician targeting for those patients. These are the remaining patients out of the 1,500 diagnosed patients we know via healthcare claims data that have carried this diagnosis within the last two years. We will have commercial availability and launch for these patients starting February 4th. Often these patients have seen a neurologist or neuromuscular specialist yet in some cases there isn't a neurologist denoted in their claims data. Instead, it is another physician such as a rheumatologist or an internal medicine specialist. Based on this category, we are thinking about 1,200 diagnosed patients who are treated predominantly by neuromuscular specialists and neurologists in our target database. Our field-based sales force will be reaching out to educate these physicians about LEMS and our newly available FDA approved treatment.

Between the patients with experience on Firdapse clinical studies, the experienced 3,4-DAP patients and LEMS diagnosed but inexperienced patients, we expect close to 300 LEMS patients into Catalyst Pathways by the end of 2019, and we're very excited about the prospect of helping that many LEMS patients and their families.

Our fourth critical success factor is rapid and comprehensive reimbursement approval by payers and insurers. This ties in with our first three priorities very well. I have already mentioned that our initial market research and work with payers to understand how they will provide coverage of Firdapse at this point. We believe it our responsibility to partner with the payer community to ensure that they are only paying for a drug that provides value to the healthcare system and a clear benefit to patients. In addition to providing them with a confirmed diagnosis of LEMS, Catalyst Pathways will be providing new Firdapse patients with free drug during their initial dose titration to help understand if they have actually obtained a therapeutic benefit before submitting forms to their payer for reimbursement. Patients who do not see a therapeutic benefit from Firdapse upon titration to the maximum dose will not be provided further free drug and claims will not be submitted to their insurance company for reimbursement. We are calling this program our My Firdapse Therapeutic Dose Program. We feel this is responsible.

As with other ultra-orphan drugs, during the initial launch period we do expect that securing reimbursement may take 60 to 90 days from the time of referral. For these patients, our free drug bridge medication and patient assistance program, PAP, will help patients access the medication and address potential gaps or lapses in therapy. We anticipate this ability and timing of securing reimbursement will improve as commercialization progresses.

By the end of 2019, we expect the majority of insurance plans will have coverage policies established for Firdapse for LEMS patients as described in our label.

Our final critical success factor has been to commit to continue listening intently to the LEMS patients and community to better understand the disease, its impact and ramifications, and to equip Catalyst to respond to their needs. We've conducted in-depth research studies, talked to rare disease and neuromuscular patient organizations, and convened patient advisory panels, asking LEMS patients to help us develop our training materials and share their personal stories with our Catalyst Pathways and field sales and Market Access teams.

We learned of their worry about lapses in treatment and put together programs to address situations that might create lapses while obtaining coverage. We understand that this disease has affected their finances and we have developed financial assistance programs designed to keep patients' out-of-pocket costs to \$10 per month or less. If necessary, we will advise them of other options on insurance coverage that might minimize their monthly premiums, and also to the availability of nonprofit organizations that might provide financial support to eligible patients.



As much as we are legally allowed, we are operating with the goal of no patient left behind. This is what motivates our efforts to engage within the LEMS community. We are really excited about making a difference.

With regards to our field-based organization, we have identified the need for a field sales team of 12 including leadership across the U.S. We have received applications from more than 150 qualified applicants, and pleased that over 80% of our positions have already been filled with excellent candidates. The sales team that will support Firdapse is comprised of professionals with an average of 19 years of pharmaceutical industry experience, and successful track records with rare orphan neurological products.

In addition, we will have five patient access liaisons who immediately connect with patients and providers once a prescription has been written. These PALs are experienced in educating patients and can effectively explain our Catalyst Pathways programs and tools, educating new patients about LEMS, their new medication and help them navigate the dose titration period as well as address the random questions that often come up during the treatment process. We have four of those five positions filled with highly experienced and passionate people who are excited about helping LEMS patients. We expect to be fully recruited and deployed for the entire sales and Patient Access Liaison team by the time of our national launch and training meeting in January.

They will join our field-based Medical Science Liaison, or MSL, team, that has been in place for the past three to four years growing from two to four this past summer and now six. The MSLs work with neuromuscular experts and clinical trials sites for all of our clinical studies in LEMS, CMS and MuSK. They are instrumental in helping physicians and patients understand the process to transition from the LEMS Expanded Access Program which will fully close at the end of March.

Reporting to Gary Ingenito, our Chief Medical Officer since 2015, these MSLs have already started communications with EAP sites and physicians on the steps involved to close down the LEMS portion of the study and allow LEMS patients to transition without lapse in their therapy while maintaining the non-LEMS parts of this Expanded Access program.

I am also pleased that Gary and his Medical Affairs Team have developed a number of peer-reviewed abstracts and publications, including the upcoming publication of the manuscript describing the results of our second Phase III study, Study 003, which should be publicly available at or around the time of our commercial launch.

So, to conclude, we are rapidly reaching full commercial readiness to successfully launch Firdapse. We have assembled a best-in-class team with specific orphan and neurology disease experience. The deployment of the field organization is underway and we have defined our initial list of neuromuscular specialists and neurologist targets who treat the approximately

1,500 currently diagnosed LEMS patients, and we will work with the physicians to identify and diagnose new patients.

Our sales, marketing and educational materials are now being finalized with the receipt of our FDA approved label, and our training materials are in the final stages of completion.

Catalyst Pathways is up and running and our care coordinators are already actively dialoguing with patients.

Our key payer account directors have been interacting with our targeted key accounts that represent most of the commercial and public payers.

And finally, our rare disease experienced exclusive specialty distribution network is in place, ready to dispense Firdapse by January 15 to experienced Firdapse and 3,4-DAP patients, and on February 4th for the remaining LEMS patients who are ready, waiting and deserving of an improved standard of care.

I will now turn the call back to Pat.

Patrick McEnany: Thank you, Dan. So let me summarize today's call. Previously, approximately 10% of LEMS patients had early access to therapy. Now all patients will have affordable access to an FDA approved medicine. We have met with the top payers in the U.S. to describe our product, pricing and programs and have encouraging and consistent responses from payers regarding coverage of Firdapse for LEMS patients. Catalyst Pathways will provide direct co-pay assistance where we can so that a patient's out of pocket cost should be nominal. Catalyst will also support independent charitable organizations that provide general support for LEMS patients.

For patients that don't qualify for these programs and are without coverage, we will provide free drug through our Patient Assistance Program, again, affordable access for all.

Physicians that we have met with have indicated if payers cover Firdapse, and if their patient is out of pocket a nominal amount, on that basis they give us high marks for a well-designed program.

We have a solid and comprehensive launch plan in place which we are currently executing on and our goal is to have 250 to 300 patients being treated with Firdapse by the end of 2019.

Lastly, we continue to invest in clinical trials evaluating Firdapse for other rare neuromuscular diseases, as well as commencing our development program for a long-acting, more patient friendly formulation of Firdapse.

We will now turn the call back to the operator so that we could take your questions.

Operator: Thank you. We'll now be conducting a question and answer session. If you'd like to be placed in the question queue. Please press star, one on your telephone keypad. A confirmation tone will indicate your line is in the question queue. You may press star, two if you would like to remove your question from the queue. For participants using speaker equipment, it may be necessary to pick up your handset before pressing star, one. One moment, please, while we poll for questions.

The first question today is coming from Charles Duncan from Cantor Fitzgerald. Your line is now live.

Charles Duncan: Good morning, Pat. Thanks for taking the question. Also, thank you for a really thoughtful and thorough overview on this go-to-market plan. You actually answered many of my questions but clearly you folks have been thinking about this deeply.

Pat McEnany: Good morning, Charles.

Charles Duncan: Good morning.

Patrick McEnany: Thank you.

Charles Duncan: My first question is along the lines of pharmacoeconomic value. You laid out some of the considerations in terms of the burden of disease and the challenges for these patients, but when you consider the key elements of pharmacoeconomic value that Firdapse can bring, would it be clinical? Would it be access, etc.? What are the main drivers of your views there?

Patrick McEnany: Charles, I'll turn that question over to Dan.

Dan Brennan: Sure. I think kind of following where you were leading, the main elements of the benefit, the pharmacoeconomic benefit is coming from the clinical aspects of the treatment. It's rapid improvements that we've seen in the studies, and we know from the patients that in many cases their disease is affecting them working. They've been also looking for a diagnosis and for effective treatments for years in cases, and so now with hopefully a quicker diagnosis and quicker to a treatment that works for them, you would avoid some of the clinical downstream effects.

Charles Duncan: Okay, that's helpful and consistent with our diligence in terms of the clinical benefit. If I could maybe transition to you, Dan, and ask you specifically about you laid out very comprehensively your five key elements for success, but I guess I'm wondering just over the course of the next year, are there any particular metrics beyond just patient access that you would like investors to focus on with which to measure your effectiveness in this rollout.

Dan Brennan: The main areas of metrics and measurement that we're looking at are going to definitely be related to those critical success factors, but, you know, they're related to adoption, certainly the number of new patient starts or new enrollment forms turned in. We're also going to be looking at the physician and provider office satisfaction in setting up this process and expectations of this process. Certainly from a market access standpoint on reimbursement, we'll be looking at the number of plans covering so the percentages of our patients' plans that are covering Firdapse. We're also going to be looking at those plans and if they're covering are they following the indication or the label or are they going more restrictive upon which we will make sure that we will go out and discuss and educate those plans about the disease and the label. We're also just going to be looking at response and resolution times for reimbursement issues.

Then there's a slew of measurements that we'll be looking from at an operational standpoint at Catalyst Pathways, including when an enrollment form is turned in, quickly making sure that it's fully complete because sometimes that's a delay for patients and the reimbursement process. We'll look at the average number of days from payers to receive information to give the approval, the coverage determination, and then again, patient and provider satisfaction with that.

Those are to us key leading indicators of success that ultimately leads up to more and more new patient starts.

Charles Duncan: Excellent. I've got to tell you I really that focus on responders for patients that are getting clinical benefit.

My last question for you is that relative to the label, you kind of brought it up. When you take a look at the label and what was granted by the agency, do you see any restrictions or any surprises? Obviously you'll market to the label but anything in particular that you'll want to specifically educate prescribers about?

Patrick McEnany: Gary, do you want to take that? Dr. Ingenito?

Gary Ingenito: Yes. Thanks, Charles. No, actually, we don't see anything restrictive in the label and feel as though it is very reflective of the data that was generated across our clinical trials.

Dan Brennan: This is Dan. I'll chime in a little bit on that, too. This won't be for the first wave of patients that have either been in our studies or on 3,4-DAP, investigational 3,4-DAP from the other company. The dosing and titration, it's going to be tricky for physicians that have never experienced this drug before, and so we have a keen eye on that. Again, it's not going to be much of an issue for the first set of patients that are coming in, but naïve patients to Firdapse and 3,4-DAP and talking with their physicians, the guidance and the language in the label, it's there but it's one thing to read it and it's another thing to actually go through that to get the maximum therapeutic benefit, and we have programs and people in place to help that.

Charles Duncan: My final question is for Pat, relative to the other indications that you're evaluating. I'm assuming you're going to be in a position to give an update early in the coming year, but how are things gone with regard to CMS in terms of patient enrollment in that trial, and interest?

Patrick McEnany: Charles, the CMS study, as we've stated in our disclosure, we expect to readout about mid-year, and the same for the MuSK-MG study. We're on track with both of those.

Charles Duncan: Sorry to interrupt. Any feedback from investigators relative to the approval of Firdapse in terms of interest in these other indications?

Patrick McEnany: I'm not sure I understand that question, Charles.

Charles Duncan: I mean clearly the approval raises the awareness of Firdapse availability. Any feedback with regard to patient interest more broadly that are affected with these other neuromuscular disorders, or too early to read?

Patrick McEnany: Yeah, it's too early to read. We have had additional investigators that have reached out to us that have patients with other rare neuromuscular diseases that they think that Firdapse could be possibly effective in treatment, and we'll continue to look at potential, other potential indications, but at this point I think it's a little too early and we do have our plan in place for 2019 to explore a couple of other indications and small pilot studies, proof-of-concept studies.

Charles Duncan: Okay. Thanks for taking my questions. Sorry for taking so much time.

Patrick McEnany: Thank you, Charles.

Operator: Thank you. Our next question today is coming from Joe Catanzaro from Piper Jaffray. Your line is now live.

Joe Catanzaro: Just a couple of quick ones from me. I was wondering maybe if you can provide a little more sort of qualitative insight into the Expanded Access Program, specifically the average duration of treatment you've seen and how that differs between paraneoplastic patients and non-paraneoplastic patients.

Patrick McEnany: Gary, would you take that?

Gary Ingenito: Sure. The duration of patients, because many have rolled over also from previous clinical trials, is over two years and some up to three years.

Regarding the paraneoplastic patients, patients had to be already treated for their cancer before being able to go onto Firdapse, so, again, since many of our paraneoplastic patients have been in remission for a number of years, their survival and length of time within the study has also been quite good.

Joe Catanzaro: Okay, got it, thanks. Then maybe just some quick sort of modelling questions. How should we think about gross to net adjustments in these first couple of quarters and maybe how that evolves over time? Then can you just remind me the royalty rate to BioMarin and then how sort of we should think about operating expenses in 2019 as the commercial launch kicks off?

Dan Brennan: Sure. I'll start with the gross to net. I think it's too early to get into specific or definitive direction here, but we'd suggest maybe start with the standard gross to net assumptions that you would see for oral orphan drugs, which is typically between 15% and 20%, and then once we start getting actual data over time with payer mix and other things, we can give you more clarity once we're in the commercial realm.

Patrick McEnany: To the second part of your question, the royalty rate for BioMarin is 14% on revenues in a given year up to \$100 million, and anything above that is subject to an additional 3% royalty, so a total of 17% max.

Joe Catanzaro: Okay, thank you. Then maybe about operating expenses in 2019 as the launch gets underway?

Patrick McEnany: We're not ready to give guidance on that. We're in the process of finalizing the budget. We actually have a board meeting next week to finalize the budget and I think then we'll be in a better position to speak more definitively to those costs for '19.

Joe Catanzaro: Okay, fair enough. Thanks for taking my question and congrats again.

Patrick McEnany: Thank you.

Operator: Thank you. Our next question is coming from Edward Nash from SunTrust. Your line is now live.

Edward Nash: Great. Thanks, guys, and again, congratulations on the approval and appreciate the detail today on the call.

Patrick McEnany: Thank you, Edward.

Edward Nash: I'm trying to back in to how many of patients you expect to be new starts on Firdapse that have not been exposed to drug previously. You've guided for 250 to 300 patients by the end of 2019. Can you remind us how many patients that you currently have are on

Expanded Access from previous Firdapse trials? Then how many that you are potentially assuming that have been receiving 3,4-DAP on emergency IND access that you think will transition over?

Patrick McEnany: We don't talk specifically to the number of patients in our Expanded Access Program. We do know based on public statements by Jacobus that they said they have a couple of hundred patients on 3,4-DAP, so we think—and this is sort of easy to back into but we think that only about 10% of the LEMS prevalence population is currently receiving Early Access to drug, so that would translate to about 300, which we're really looking at as our critical success factor one to convert those patients during Year 1, 2019.

Edward Nash: Okay. Okay, and then you had mentioned that of the 1,500 patients that are diagnosed out of the total prevalence, I might have missed this—how many physicians are needing to be targeted to be able to address basically those 1,500 patients?

Dan Brennan: Our target universe that covers those 1,500 physicians, and perhaps a little bit more because the claims data is not perfect, as many of you know; it's much different than prescription data. We have a target universe of about 1,200 or 1,300 physicians that are really for the most part neurologists and neuromuscular specialists.

Edward Nash: Okay, and the 1,200 to 1,300 is assuming those 1,500 and then above and beyond that?

Dan Brennan: Well, we do believe that those 1,200 to 1,300 physicians are probably seeing at some point in time some of these undiagnosed or misdiagnosed patients also, so we might be able to help on that end as well with other patients that they see but they just hadn't flagged yet with a diagnosis.

Edward Nash: Okay, great. That's helpful. Thank you, again, and congratulations.

Patrick McEnany: Thank you, Edward.

Operator: Thank you. Our next question today is coming from Leland Gershell from Oppenheimer & Company. Your line is now live.

Leland Gershell: Hey, good morning, Pat and team. Thanks very much for having this very comprehensive call.

Patrick McEnany: Good morning.

Leland Gershell: A couple of questions for me, really for Dan. You had run through the breakdown of coverage distribution amongst LEMS patients, commercial versus Medicare, Medicaid. Was that among all LEMS patients or is that among those who have some sort of plan

and may exclude perhaps a number of those who are missed by the system or fall through the cracks?

Dan Brennan: That's our understanding from our data and our data comes from a lot of different sources; a bit from our understanding of the patients in clinical studies but then also from the claims data, and that's looking at LEMS patients only, or LEMS-diagnosed patients only.

Leland Gershell: Okay. Then with regard to transitioning those who are on either Firdapse or 3,4-DAP, obviously those are the patients who since they're taking it you want to get them over and not have a lapse, so they may be the sooner ones we see come onto commercial Firdapse. Do we have or can you talk about what the coverage nature is among those patients? In other words, if we could think about what fraction of patients who are on those plans will be coming on drug with commercial coverage versus those who may not be? Just so we have a sense of maybe what timelines and so forth for reimbursement purposes, the low-hanging fruit, so to speak, of patients who will be coming onto commercial drug?

Dan Brennan: Right. Well, on one hand the good news is a lot of those patients, and quite honestly many of these patients and sites have already reached out to us to begin the process of transitioning which is great. The challenging part of course at the beginning of any approval and launch is when do the insurers actually get the coverage determination made. They have difficulty at the beginning of a new drug coming onboard. We see this varying, quite honestly, anywhere from immediate to 180 days in some cases.

We're more expecting an average of 60 to 90 days in the early months of launch, so call it the first half of the year that patients coming onboard will have an average of a delay of 60 to 90 days while the plan tries to understand, "Okay, let's put it in our system. Let's run it through." In some cases, if it's Medicare, for example, it's not on their formulary for all of 2019, but they do have an exception policy, an exception process for new drugs, and so that takes longer to run through appeals and such.

We're looking at an average of 60 to 90 days for these patients in the first half and probably the better part of next year. And that will improve over time.

Leland Gershell: Okay. Great. That's very helpful. Then with the aspiration to enable access by those other—I think the number was 1,200 or so patients who are known to have LEMS but may not be receiving any form of therapy, kind of what do you see as the hurdles there? In other words, presumably many of these patients are aware or have been aware of the other products and also your program. Now that there's an approved drug, have you been getting inbound calls? Have you—kind of if you could give us sort of some light on the ease to which you might see getting access (inaudible).



Dan Brennan: Yeah, so I think this is one of the reasons we put together this My Firdapse Therapeutic Dose Program. When they come in, they'll actually—if they and their physician are interested, they'll have access to free medication during the titration period where we can help them and their physicians through titration, understand the therapeutic response, and then ultimately we provide that information over to payers, and this is kind of again the program that's insuring almost a number of needed to treat of 1 which when we talk to the payers they love that; when we talk to physicians they like this. We have a program in place to help these naïve patients get the product, have access to it, try it out, go through this process of helping them with the dose titration, and then ultimately if and when they respond the payers have a much easier time in accepting that coverage determination.

Leland Gershell: Okay. Okay, great. If I may, just one last question. With regard to the My Firdapse Therapeutic Dose Program, which is a program that I think is going to enable patients to stay on therapy provided there's some sort of indication that they're getting a form of benefit, I just wanted to see if you could kind of run through the particulars there of how that's administered, and also what the stringency of benefit is? In other words, is it anything to do with the label or the clinical data, or is it more based on patient and physician interpretation of how the patient has been doing on the drug?

Dan Brennan: Well, there's three main parts to it. Number one, it starts with a confirmation of a LEMS diagnosis, so ensuring the payers that there's been an antibody test or an EMG test that confirms LEMS. The second part is then free medicine for the titration period because payers never like paying for medicine during titration, so we're providing that for free as they come up to try to get the therapeutic benefit, and then to establish the therapeutic benefit, it's actually a question that was in our clinical studies called the SGI or Subject Global Impression. So, we asked that question and understand whether or not the patient on that scale is receiving a therapeutic benefit at all. If the answer to all those is yes and things are progressing, then that patient's information gets passed over to the payer.

Leland Gershell: Understood. Okay, great. Thanks very much for taking my questions.

Patrick McEnany: Thank you, Leland.

Operator: Thank you. The next question today is coming from Scott Henry from ROTH Capital. Your line is now live.

Scott Henry: Thank you and good morning. Pat, just a couple of questions.

Patrick McEnany: Good morning.

Scott Henry: First, for clarification and I think I've got this, but when you talk about 10% of patients being on therapy, as you basing that off of the 1,500 number or the 3,000 number? It sounds like it's the 3,000.

Patrick McEnany: Three thousand number.

Scott Henry: Perfect. Then another question is what do you make of compounding pharmacies? Do you think that they contribute to this population? Is that sort of a shadow group of patients? I would expect with the orphan drug designation and approval they probably shift over to you as well. Any comments on that category?

Patrick McEnany: Scott, I'll let Steve answer that, but I can tell you that since the Drug Quality and Security Act was passed several years ago, I think it's minimal, but Steve can speak more specific to today's environment.

Steve Miller: Yes. We are actually interacting on a regular basis with close to 100 experts and we almost never run into situations where patients are receiving compounded drug. To expand on what Pat said, under Section 503A of the Food and Drug Act, it prohibits compounders to compound for individual patients, for preparing compounded drugs that are essentially copies of approved commercially available drugs under regular or inordinate amounts. Typically, the only compounding they're allowed to do under that section is compounding for a clinically different version that's medically necessary, like a different route of administration.

For larger scale compounding, which by the way we have never seen for this drug, that would be registered outsourcing facilities under Section 503B and those facilities are not able to use amifampridine in compounding unless the drug is nominated and placed on the FDA's Bulk List 1, and amifampridine has actually been nominated twice for that and has not been put on the list, and it's currently not on the list, therefore compounding amifampridine by any outsourcing facility is actually illegal.

Scott Henry: Okay, great. Thank you for that, that color. Final question. I know you kind of set a target of roughly 300 patients by year-end 2019. Can you just talk just subjectively and briefly about the cadence of when you may accrue those patients, and then how we should think about the time lag of booking revenues for those patients? Just any kind of subjective comments would be great.

Patrick McEnany: Scott, I'll let Dan answer that, but as we've said without great experience yet in adoption rates and uptake, we look at the patients that are on 3,4-DAP or in our Expanded Access Program as, again, our critical success factor 1 for this year and so – and as Dan said previously, initially it's going to take time before they are revenue producing, if at all. Again, some of those, as you know, will basically be on free drug forever because they just fall through the crack.

Dan, you want to elaborate on that?

Dan Brennan: Yeah, and having worked in a number of different rare disease areas, I expect the same thing will happen here is that there's an initial pent-up demand from all those patients that are aware and the physicians that are aware, and in this case those are the people that have been in our Firdapse clinical studies or this 3,4-DAP investigational drug. Then once they kind of come in – and they'll come in relatively quickly in the first two or three, four months, but then it becomes hard going because the naïve patients whose physicians or themselves have never heard of this, it's a rare disease. The physicians don't think of the one or two patients on a regular basis – “Oh yeah, I have that LEMS patient,” that maybe it's—it's just a longer road. It really is kind of a lot of patients in in the first three, five months, as Pat mentioned, then we have that delay in coverage that I mentioned earlier for them to actually get to reimbursed paid drug, and then over time it's just our efforts with educating physicians, bringing stronger awareness to patients as well as the physicians, and trying to put it top of mind for them with a patient that really only comes into their office one or two times a year.

Scott Henry: Okay, great. Thank you for taking the question.

Patrick McEnany: Thank you, Scott.

Operator: Thank you. We've reached the end of our question and answer session. I'd like to turn the floor back over to Pat for any further or closing comments.

Patrick McEnany: Thank you for your participation on today's call. Before ending this call though I'd like to take a moment and thank the LEMS patients, their families and the investigators who participated in our comprehensive clinical development program. We really appreciate their support in the development of this important new treatment.

I'd also like to recognize the FDA for their professional, fair and proactive review process.

Lastly, but certainly not the least, I'd like to recognize our Catalyst team. They worked very hard for many years with an exceptional degree of focus, dedication and enthusiasm. It is very gratifying for all of us here at Catalyst to reach our goal of bringing Firdapse to the patients with LEMS who desperately need it.

We look forward to providing you with additional updates on Firdapse and the rest of our business throughout the year. Thank you.

Operator: Thank you. That does conclude today's teleconference. You may disconnect your line at this time and have a wonderful day. We thank you for your participation today.