# NPS Pharmaceuticals' CEO Discusses FDA Approval and Commercial launch of Gattex (Transcript)

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About: NPS Pharmaceuticals, Inc. (NPSP)

**Operator**

Good day, ladies and gentlemen, and welcome to the NPS Pharmaceuticals Conference Call. My name is Caris and I will be your coordinator for today. At this time, all participants are in a listen-only mode. Later, we will conduct a question-and-answer session. (Operator Instructions) As a reminder, this call is being recorded for replay purposes.

And I would now like to hand the call over to your host for today, Susan Mesco, Senior Director of Investor Relations and Corporate Communications. Please proceed.

**Susan Mesco**

Thank you, Caris. Welcome to today's conference call to discuss the commercial launch of Gattex. Before we start, let me remind you that today's call 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 our website for information concerning the risk factors that could affect the company.

Joining me on today's call are Dr. Francois Nader, our President and CEO; Eric Pauwels, our Chief Commercial Officer, and other members of our senior management team.

I will now turn the call over to Dr. Francois Nader.

**Francois Nader**

Thank you, Susan. Good afternoon and a very Happy New Year to everyone joining us on today's call. This was a very exciting holiday season for the NPS team and we were thrilled to close 2012 with an outstanding accomplishment with the U.S. approval of Gattex. After completing the most comprehensive clinical development program to-date in short bowel syndrome, it is very gratifying to finally be able to deliver this much needed therapy to patients.

For nearly 40 years, short bowel syndrome patients have relied on parenteral support to receive the nutrients and fluids that they need to live, but have risked serious life-threatening complications along the way. With the approval of Gattex, the lives of these patients can change.

Gattex is the first and only long-term targeted therapy that addresses the underlying issue of short bowel syndrome, which is mal-absorption. As you know, short bowel syndrome patients can be hooked to an intravenous line 10 hours

each week, and nearly a quarter of patients achieved three or more infusion-free days each week. These data could translate into anywhere from 50 days to 150 days of independence from parenteral support each year.

The interim results of our two years study of Gattex in short bowel syndrome showed that 72% of patients achieved at least a 20% or greater reduction of parenteral support, after a year of Gattex treatment, with a 40% mere reduction in weekly volume. Further, what is especially exciting is that many patients achieved complete independence from parenteral support, while on Gattex, after being chronically dependent on PN, for up to 18 years.

Freedom for – from parenteral support allows patients to experience the simple activities that many of us take for granted by joining family functions, traveling from home for a weekend or just a simple night of uninterrupted sleep and that importantly completely eliminate the risk inherent to parenteral support. We are very pleased with the outcome of our label negotiations with FDA and as you probably know it by now, Gattex is indicated for the treatment of adult patients with short bowel syndrome, who are dependent on parenteral support. And it is approved for clinic use, making Gattex the first long-term treatment for short bowel syndrome. As such the label provides the flexibility for physicians to determine the best time to initiate Gattex therapy for their patients.

With respect to safety, the warnings and precautions are consistent with the non-pharmacology of Gattex, as well as the complications associated with short bowel syndrome and its underlying etiologies and certain clinical assessments are defined to ensure the continued safe use of the product.

We believe the risk evaluation and mitigation strategy or REMS program is very practical and aligns very well with our commitment to ensure that Gattex is used appropriately and that patient initiations are successful and sustainable. NPS and the FDA recognized the need and importance of conducting a voluntary long-term patient registry, which has been defined as a post-marketing requirement. We believe the registry will be instrumental in gaining a better understanding of Gattex for the long-term treatment of short bowel syndrome.

I would like now to address the commercial opportunity that Gattex represents. We previously reported from the literature and our own prevalent studies, an estimated 10,000 to 15,000 adult short bowel syndrome patients, who are chronically dependent on parenteral support 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 using top-down prevalence estimates for rare diseases, because of the small number of patients and the even smaller sample size used in prevalent studies.

The recent month, we initiated a bottom-up and added this to more precisely quantified addressable population. Important sources of patients were those managed by the top five national home infusion companies. Additional patients were identified through our website shortbowelsupport.com, through previous clinical trial participants and information gathered from our MSLs.

We also worked with various patient advocacy groups that support SBS patients or its underlying etiologies. Based on our findings, short bowel syndrome qualifies as an ultra-orphan disorder with an estimated Gattex addressable population of 3,000 patients to 5,000 patients in the U.S. While the addressable market is less than our initial prevalence estimate, we were very pleased with the results of our pricing research.

More recently, our commercial team commissioned a rigorous pricing and access study, with an internationally recognized consulting firm, who has specific experience in pricing multiple successful ultra-orphan products on the market today. Many factors were considered in establishing the price including the fact that Gattex is the first and only long-term approved therapy for short bowel syndrome, which is a life-threatening disorder.

The value Gattex provides to patients by improving the absorptive capacity of their intestines and reducing or completely eliminating the need for parenteral support, payors' understanding of the direct and indirect burden of short bowel syndrome, the limitations and costs of current supportive care options and the need for an effective long-term

addressable patient population and the ultra-orphan status of Gattex and last, but certainly not least, NPS investment in the most comprehensive research and development program conducted to-date in short bowel syndrome.

Consequently, and based on these considerations, we have set the annual list price of Gattex at $295,000. At this price level and given the expected patients' adherence and compliance rates of 70% to 80%, we expect Gattex to achieve annual peak revenues well in excess of the $350 million per year that we previously expected. Our pricing strategy will go hand-in-hand with a focus on ensuring broad and sustainable coverage from both private and public payors.

The NPSP did not spare any efforts over the last couple of years to ensure comprehensive commercial readiness and are undoubtedly ready to successfully launch Gattex in the U.S.

At this time, I would like to invite Eric Pauwels, our Chief Commercial Officer to comment on our launch plans. Eric?

**Eric Pauwels**

Thank you, Francois, and good afternoon to everyone on today's call. One of our very first elements of ensuring the successful commercialization of Gattex was to assemble a truly world-class team of passionate industry veterans. These individuals came to NPS from leadership roles at rare disease biopharmaceutical companies and many with specific expertise in successfully launching orphan products.

Gattex launch-readiness focused on five key areas. Building our field-based organization; physician targeting; patient identification; NPS Advantage, our concierge-like patient support program; and ensuring rapid and comprehensive market access.

With regard to our field-based organization, our leadership team has been in place for a couple of months and includes three key account directors and three regional business directors. For our sales organization, we are extremely pleased with the level of interest and talent that we've attracted, which went up to 24 area business specialists for the approval of more than 250 qualified applicants. The sales team who will market Gattex is now comprised of seasoned professionals with an average of 15 years of pharmaceutical industry experience and a successful track record with injectable orphan biologics.

Our field force will be fully deployed in February, after we conduct our national sales meeting. In addition, NPS Medical Affairs has deployed a team of eight medical science liaisons, who have been building awareness amongst SBS thought leaders for over a year and are well positioned to educate the medical community on the clinical value and profile of Gattex.

We are pleased that the launch of Gattex will be supported by an impressive number of peer-review publications, including the results of our Phase III STEPS study, which appeared in the December issue of Gastroenterology.

Our next strategic objective was to identify physician targets. As we mentioned on our last call, we developed an initial target list comprised of approximately 6,000 physicians who are practicing in roughly 1,000 institutions that includes centers of experience and large GI practices. These potential prescribers will be better refined once our field force begins calling on them individually.

Now, switching to patient identification. Given the ultra-orphan status of short bowel syndrome, along with the fact that this is a condition of a condition, without a formal support of organization, patient identification is a critical strategic objective. Despite the limitations of finding patients in advance of having an approved product, I'm very happy to report that we have identified over 1,000 unique short bowel syndrome patients who are eligible for Gattex therapy. Now that we have secured approval, our patient identification activities will increase as our field organization will communicate the value of Gattex to physicians and other stakeholders and identify additional eligible SBS patients.

providers with individualized support including personalized case management, reimbursement assistance, product shipment scheduling, time-based deployment, product refills and prescription renewals. We're also leveraging the relationships that we have established with a number of patient advocacy groups such as the Oley Foundation, of course, to play an important role communicating the availability of Gattex to their members and disseminating educational materials.

The last area I would like to cover is the importance of ensuring their access to Gattex. Our market research shows that most short bowel patients have insurance coverage, and it's approximately 60% covering by commercial plans versus 40% were covered by government and publicly insured. And, the majority of the product is being Medicare.

Our key account directors have deployed – have been deployed since October and are targeting the 75 key payor accounts that cover up to 80% of the U.S. insured population. Government have our label approved, we've been – we will put the final touches on our payor communication and educational materials, including the AMCP dossier. These tools characterize the SBS burden and the value of Gattex.

Based on the indications, we expect payors to provide broad access to Gattex. In our market access studies, payors clearly recognized the high unmet medical needs of short bowel syndrome, as well as the therapeutic improvement that Gattex represents over current treatment options. The value proposition is also notably strengthened by the many patients who achieved complete independence from parenteral support with long-term Gattex therapy. All payors indicated they would cover Gattex with an anticipated tier placement and restrictions fairly typical of other ultra-orphan products.

Here also our care coordinators are ready to help stakeholders, especially patients, successfully navigate the reimbursement hurdles such as prior optimizations and other restrictions. In addition, we have established a comprehensive patient assistance program.

First, our patients with commercial coverage, copay assistance will be coordinated through NPS Advantage to minimize or even eliminate our pocket burden. We also utilize third-party foundations for patients covered by public plans. And lastly, to those rare cases in which patients do not qualify for assistance or to bridge gaps in coverage, we offer a free goods program. As with other ultra-orphan drugs, during the initial six months to 12-month launch period, we expect that securing reimbursement to take 90 days to 120 days from the time of referral. We anticipate this period will improve as the commercialization progresses.

As a reminder, Gattex will be reimbursed as a pharmacy benefit or Medicare Part D and will be dispensed by our

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