Spinraza, the first FDA-approved drug that corrects the underlying cause of SMA, is now available commercially in the United States. Stanford has over four years of experience using Spinraza and we are excited to offer it to people of all ages. Stanford was the first to treat an infant in 2013, and since FDA approval of Spinraza in December 2016, we completed 3 of the first 6 commercial treatments of children and we treated the first adult. The Stanford SMA program was at the forefront of clinical trials for Spinraza, and is involved with the development of additional motor neuron treatments. To learn more about our research program, or to participate in studies, please register on our research program mailing list by emailing NeuromuscularResearch@stanford.edu and requesting a database consent form.

Individuals who want Spinraza can receive it as part of their overall care at either the pediatric or adult Stanford Neuromuscular Clinics. Stanford has weekly multi-disciplinary clinics for infants, children and adults with SMA, and now incorporates Spinraza into the comprehensive care of these patients. Both the Lucile Packard Children’s Hospital (LPCH) Pediatric Neuromuscular Clinic and the Stanford Neuroscience Health Center (SNHC) Adult Neuromuscular Clinic have accomplished teams of physicians, nurse practitioners, therapists, social workers and other providers who are experienced in the care and support of individuals affected by SMA.

The Stanford Neuromuscular Spinraza Treatment Program includes the following elements:

- To receive Spinraza at Stanford, all individuals must be registered at, and receiving their care at, either the Stanford pediatric (LPCH) or adult (SNHC) Neuromuscular Clinic (see contact information below)
- The Stanford Neuromuscular team will work with each patient and family to obtain prior authorization from the patient’s insurance, which is required before Spinraza can be administered
- Spinraza injections follow a specific schedule. Patients receive 4 injections in the first 2 months as loading therapy. Maintenance therapy consists of one injection every 4 months.
- Because Spinraza takes time to work, to maximize its benefits patients must also receive clinical support meeting SMA care guidelines, as is provided at Stanford or similar multi-disciplinary neuromuscular centers.
- All Stanford patients who receive Spinraza, whether at Stanford or another facility, are scheduled to return to our comprehensive clinic every 4 months to monitor their response to the drug and adjust their management

Contact Us

LPCH Pediatric Neuromuscular and SMA Clinic – New patients call: (650) 723-0993, select option 3 then option 2
LPCH Pediatric Neuromuscular and SMA Clinic – Return patients call: (650) 723-0993 option 3 then option 1
SNHC Adult Neuromuscular and SMA Clinic – New patients call: (650) 723-6469 option 2. Have your current physician submit a referral and any relevant medical records such as genetic testing results. To aid in processing, your physician should state that you are interested in Spinraza treatment. Referrals can be faxed to (650) 723-6002. Once processed, our New Patient Coordinator will contact you to schedule an appointment.
Those with SMA who are 21 years of age or older are welcome to receive comprehensive care in the Stanford Adult Neuroscience Health Center (SNHC) Neuromuscular Clinic. Patients aged 16-21 years of age who want to receive Spinraza treatment at SHNC, even though they are receiving their on-going care at LPCH, are also welcome to schedule an appointment in the SNHC Neuromuscular Clinic. If you want to be seen at the SNHC Adult Neuromuscular Clinic please contact us at the number below.
SNHC Adult Neuromuscular and SMA Clinic – Return patient scheduling: (650) 723-6469 option 1 and ask to speak with the Neuromuscular Patient Care Coordinator to schedule your Spinraza evaluation appointment. As our call volume is high, you may be asked to leave a message and your call will be returned within 24 hours.
Stanford SMA Research Program - If you are interested in participating in SMA research at Stanford, or want to be notified about on-going research possibilities, please register in the Stanford Neuromuscular Database by communicating with the Stanford Neuromuscular Program. (650) 725-4341; email address NeuromuscularResearch@stanford.edu

Pediatric Patient Appointments

1. If your child has previously been seen at LPCH Neuromuscular Clinic but has not had a follow-up visit in the last 6 months, please schedule an appointment by calling (650) 723-0993, select option 3, then option 1.
2. If your child has not been seen at the LPCH Neuromuscular Clinic, please call the new patient scheduler to make an appointment at (650) 723-0993, selecting option 3 and then option 2; have your doctor fax referrals to (650) 721-2884. Please have your doctor indicate that you are interested in Spinraza for more accurate processing.
3. During your appointment, ask about Spinraza treatment for your child or alternate research options; discuss assistance options from Biogen Support Services; complete the Spinraza Start Form from Biogen (given to you in clinic).
4. Tests needed to establish eligibility for Spinraza can be performed at your clinic appointment. These tests include: blood work, urine tests, cardiac tests, X-rays, etc.

Adult Patient Appointments

1. If you have previously been seen at the SNHC Neuromuscular Clinic, but have not been seen in the last 6 months, please schedule an appointment by calling (650) 723-6469 and select option 1.
2. If you have not previously been seen at the SNHC Neuromuscular Clinic, have your doctor fax a referral to (650) 723-6002. Please have your doctor indicate that you are interested in Spinraza for more accurate processing. You may call the New Patient Coordinators at (650) 723-6469 option 2 to schedule.
3. During your appointment you can expect to receive more information about Spinraza. Your visit may include a consultation with one of our neuromuscular MDs or NP, baseline physical therapy assessment, baseline breathing test, labs, and imaging of your spine. Treatment is dependent on our ability to access to your spinal canal through your lower back, which can be impacted by spinal fusion.

General information about drug treatment

About the drug:
How often does the drug need to be injected?
The drug is administered at Day 1, 15, 30, and 60. Afterwards patients must come back every 4 months to receive a follow-up injection of the drug.

How much does the drug cost?
The wholesale cost for the drug alone is $125,000 per dose. This does not include the cost of neurology clinic visits, routine testing and procedures for injecting the drug. Furthermore, each institution negotiates its own contract with each insurance company to cover the costs of drugs, so that specific arrangements to cover the costs of the drug need to be worked out individually for each patient.

Does insurance cover it?
We will obtain prior authorization from insurance before administering the drug. Some insurance companies have covered the test so far, but we do not yet have a list of known approvers, and anticipate that this will take several months to become clear. We also expect that some insurance companies that have announced limits to their approval of the drug will modify their decisions.

Does it work? What will it do?
SMA results from the loss of a specific gene, SMN1 (survival motor neuron 1) in all patients with SMA. This gene produces the SMN protein, which allows nerves that control muscles to function properly. All patients with SMA also have at least one copy of a back-up gene, the SMN2 gene, which can produce a small amount of SMN protein. SMN2 does not perform very well so does not make enough of the SMN protein for the nerves to be healthy. Spinraza targets the SMN2 gene to help it make more SMN protein and improve the health of the nerves that control muscles.
We do not have as much data on Spinraza’s effects in adults as we do in children, but we do have results that show it improves nerve function in patients with all types of SMA (SMA1, SMA2 and SMA3) who are aged 0-20 years of age. Because of the dramatic effects of Spinraza seen in tissue from patients, as well as its dramatic and consistent effects in patients with all types of SMA, the FDA approved the drug for all patients with SMA without any restrictions regarding the type of SMA, the age of the patient or the severity of the patient’s disabilities. Our primary goal in using Spinraza is always to assure that the patient’s course will stabilize, and that no additional function will be lost, but in some instances there is also some degree of functional improvement.

**How is it administered?**
The drug is injected into the spinal fluid in the lower back in what is called an “intrathecal injection”. This involves a lumbar puncture, or spinal tap, with injection of medication directly into the spinal fluid. The injection is very similar to the type of injection that many people get to relieve pain in the back (like “epidural” anesthesia for childbirth). The lumbar puncture and drug injection can take up to 2 hours from start to finish.

**Is anesthesia involved?**
Most adults tolerate the spinal injection easily with a local injection of lidocaine (Novocain), so that no other anesthesia or sedation is needed. Anesthesia may be used for some children receiving Spinraza depending on their age, physical strength, respiratory status, and other variables; for example, many children between the ages of about 2 years old and 12 years old are not able to lie still during the brief injection, so that anesthesia may be used to assure that the drug all goes into the spinal fluid.

**Does the procedure hurt?**
There is an initial burning sensation during the lidocaine injection and a feeling of pressure during the procedure. There are many nerves in the lower back, so it is not uncommon to bump a nerve during the injection, which causes a brief twinge of pain that goes away quickly. Most patients do not feel this is a particularly painful procedure. Some patients (usually <10%) can develop a headache after the procedure. If a headache does occur it usually goes away on its own but can be effectively treated if necessary.

**Can patients with spinal curvatures or rods receive the drug?**
Patients with spinal curvatures or rods are eligible for treatment. Each case will be reviewed individually to determine if a route can be identified in the lower back by which the injection can be given. We are still working with colleagues at Stanford and in SMA centers around the world to optimize treatment options for all patients.

**Can patients that are ventilator-dependent receive the drug?**
Yes, as long as the medical team believes it will still be safe to undergo the treatment.
Are there any side effects? Is it safe?
Although no significant side effects have been seen with Spinraza, we are still working with Biogen and the FDA to monitor patients so that we can definitively identify any potential side effects. The potential side effects of Spinraza are listed on Biogen’s website here: https://www.spinraza-hcp.com/?cid=ppc-ggl-spinrazahcpnowapproved-lm-644-spinrazahcpnowapproved&gclid=CLf9oIv4ztECFU9efgodT5QOaA

Why do I have to do physical therapy assessments? Can’t I just get the injection?
Physical therapy assessments are important to your health care, and also allow us to see how the medication is affecting you as an individual. Spinraza is not a “magic wand” and cannot simply eliminate the effects of SMA that have developed over months and years. However, the potential benefit of Spinraza in stabilizing each person’s abilities depends on our being able to identify any changes that develop, and then optimizing positioning, support, functioning and on-going therapy so that the benefits are maximized. We recommend that all patients receiving Spinraza return every four months to a dedicated clinic of SMA experts (physicians, therapists, nurses and practitioners, such as the Stanford Neuromuscular Clinic) to assure that the effects of this treatment are as beneficial as possible. Insurance companies may require that evidence of benefit from the drug be submitted in order to receive authorization to continue Spinraza after the first year, which is another reason for the follow-up in clinic for evaluation by the Neuromuscular Team.
**Do I need to have had genetic testing done?**
Yes. Spinraza will only benefit patients who have an absence of the SMN1 gene, and have one or more copies of the SMN2 gene. If you have had testing done previously, please bring a copy of the original report to your visit if it is not already part of your Stanford medical record. This is important because sometimes there are other disorders that can mimic SMA, but are genetically different. If you have not had genetic testing done previously, because it was not covered by insurance, please let us know. Now that there is an approved treatment for SMA, we can appeal to your insurance company and expect that we will get approval for the testing very quickly. The Stanford Neuromuscular Genetic Counselor is available to help with this process. We expect to be able to get genetic results within a matter of days, or at most a few weeks, so it shouldn’t slow up the process and is essential for us to get Spinraza approved.

**Are younger patients given a higher priority?**
Newly diagnosed infants with SMA1 have shown the most dramatic benefits of the drug, so they will be treated as soon as possible. We do not expect a large number of these patients, and expect to simply work them into our existing treatment schedule, so do not expect that they will impact the order and schedule of treatment for other (older) patients with SMA. Other than infants, we expect Spinraza to benefit all patients, making it impossible to determine who will benefit more.

**How long will it take to get in for treatment?**
The drug injection programs in the Stanford pediatric and adult clinics both proceed at their maximal pace. We are currently unable to predict how long it will take to get treated, based on individual insurance issues, but we are dedicated to providing information so that you can see our progress as we work to get the treatment approved. Treatment may be delayed for patients who require a surgical intervention.

**Can people with all types of SMA receive the drug?**
Yes – as far as Biogen, the FDA and Stanford are concerned. Based on the data presented to the FDA, Spinraza has been approved for all patients with SMA irrespective of the type of SMA (SMA1, SMA2, SMA3 and SMA4 are all eligible for treatment), the age of the patient (infants, children and adults are all eligible), or the degree of disability (ambulatory and non-ambulatory individuals, those who require ventilatory support, and those who have had spinal surgery are all eligible). Each insurance company has the right to decide if the patients it covers will be treated or not, but there are no restrictions placed by the FDA, Biogen or Stanford.
**About treatment at Stanford:**

**What insurance is accepted at Stanford?**
Stanford accepts patients covered by most major insurance companies. If Stanford is “out of network” for your policy, there is also a possibility that your insurance would approve this specialized treatment at Stanford. The Stanford (LPCH and SNHC) clinic staff can help you determine whether your appointment at Stanford and any subsequent treatment would be covered by your insurance.

**Can we come from out-of-state for treatment?**
Yes, as long as your medical insurance is accepted at Stanford. Keep in mind that the first few months have more frequent injections (Day 1, 15, 30, and 60), and that we expect all patients treated at Stanford to continue their follow-up care at Stanford.

**Can we come from outside the USA for treatment?**
We are currently not set up to treat patients from outside the USA. We are open to developing this on a case-by-case basis, but in general patients wanting to receive on-going Spinraza treatment at Stanford will need to move to the U.S.A.

**Are initial pre-treatment exams needed?**
Yes. Each patient will be seen for a pre-treatment clinic consultation by the Stanford Neuromuscular Team. This will include evaluations by the neuromuscular neurologist or nurse practitioner, physical therapist, and respiratory therapist. Other disciplines are also available as part of our multidisciplinary clinic as needed including occupational therapist, speech and language therapist, respiratory therapist, pulmonologist, genetic counselor, social worker and nurse coordinator. The appointment will include the exam, labs, and spine imaging required to establish eligibility for Spinraza treatment. Once the drug is authorized by the insurance company, patients will be contacted to set up a treatment schedule.

**Is this an in-patient or out-patient procedure?**
All drug treatments will be provided by an outpatient procedure. The entire visit should last no more than 2 hours, though might take slightly longer if general anesthesia (only commonly used for children 2-12 years of age) is required.

**What are the age ranges for Stanford’s Pediatric (LPCH) and Adult (SNHC) Neuromuscular Clinics?**
We follow pediatric patients at LPCH until age 21 years so that we can optimize interaction with California Children’s Services, which is provided to neuromuscular patients less than 21 years of age.

Most patients at the Stanford’s SNHC Adult Neuromuscular Clinic are 21 years of age or older, though individuals 15 years or older can easily be seen at SNHC if desired.

For patients desiring Spinraza treatment at Stanford: 1) all patients under 16 years of age will
be treated at LPCH; 2) all patient 21 years of age or older will be treated at SNHC; 3) patients aged 16-21 years can receive Spinraza at either LPCH or SNHC, whichever hospital can accommodate them earlier and more easily. If desired, patients aged 16-21 years who receive their ongoing care in the LPCH Neuromuscular, can receive their Spinraza injections at SNHC if that is a better option, and would be expected to continue receiving their clinical care at LPCH.

*If I start drug treatment elsewhere, can I still get treated at Stanford?*
Yes. We encourage all our patients to continue receiving neuromuscular care at Stanford even if you receive Spinraza at a different institution. We recommend that we continue to monitor your condition in Stanford Neuromuscular Clinic at visits every 4 months while receiving Spinraza, at which we would undertake our standard neurological and therapy assessments to measure symptom progression or regression. Results of our evaluations every 4 months will help us adjust ongoing management, treatment and therapy plans to optimize benefits of Spinraza and maximize functional capabilities.
We strongly emphasize, in order to optimize function and control the effects of SMA, that regular comprehensive care be maintained at an established multidisciplinary neuromuscular clinic, such as either the pediatric or adult Stanford Neuromuscular Clinic. Optimizing your health and neuromuscular function remains of great importance, whether or not you are on Spinraza.

Will Stanford continue to refine and improve their Spinraza Policy?
Of course. We are eager to get feedback about what works and what could work better regarding all aspects of our SMA and Neuromuscular Program. This is a very active time for the neuromuscular field, and we remain committed to bring as many novel treatments for SMA as possible to the Northern California and greater SMA communities. Please feel free to contact us so that we can continue to improve our program and our interactions with the SMA patient and provider communities. We are committed to doing all that we can, as well as we can, as soon as we can to combat SMA and related disorders. Thanks for your help.