Summary of Results for Laypersons

Astellas is grateful to the women who took part in this clinical study. Thank you.

What was the Study Called?

A Phase 2a, Randomized, Double-blind, Placebo-controlled, Parallel-group, Proof of Concept Study to Investigate Efficacy, Safety, Pharmacodynamics and Pharmacokinetics of ASP6294 in the Treatment of Female Subjects with Bladder Pain Syndrome/Interstitial Cystitis. This is also known as the SERENITY study.

Why was this Study Needed?

Interstitial cystitis and bladder pain syndrome are conditions that cause bladder pain. And sometimes they cause pelvic pain associated with symptoms related to passing urine. The pain ranges from mild discomfort to severe. The bladder expands until it is full. It then signals the brain that it is time to urinate. With these conditions, signals get mixed up and a person feels the need to urinate more often. And the volumes of urine are smaller than what most people do. There are few effective treatments for these conditions. Therefore, there was a need to study new treatments for bladder pain syndrome with interstitial cystitis. ASP6294 is an experimental medicine given as an injection beneath the skin.

This study was conducted in women with bladder pain syndrome with interstitial cystitis. They received either ASP6294 or placebo as injections under the skin. The section below describes what placebo is. The study looked how the patient's average daily pain changed from before the study medicine started until after 12 weeks. It was also important to find out what unwanted effects these patients had from the study medicines.

The study started in September 2017 and ended in March 2019. When the study ended, the sponsor of this study (Astellas) reviewed all the study information and created a report of the results. This is a summary of that report.

What Kind of Study was this and Who Took Part in It?

This was a "double-blinded" study. That means that the patients and the study doctors did not know who took which of the study medicines. This helps make study results fair and unbiased. One of the study medicines was placebo. A "placebo" is a dummy treatment that looks like a medicine, but does not have any medicine in it.

Women 18 years or older were included in the study. They had bladder pain syndrome with interstitial cystitis for at least 2 months before the first study visit. In the past, the women had at least 2 or more treatments for their bladder pain that failed to help them. The women reported they had average daily pain of at least 4 and up to 9 on a pain scale of 0 to 10. In the scale, 0 is no pain and 10 is pain as bad as you can imagine.

During the study, the study doctor did a check-up of the women at several study visits. Starting at the first visit, women recorded their average daily pain and their worst pain in an electronic diary for 2 weeks. The electronic diary was reviewed on the second visit to see if

the women could remain in the study. To remain in the study, their average daily pain was at least 4 and up to 9. And the pain did not change by more than 4 points day after day.

Women remaining in the study were picked to receive 1 of 2 study medicines by chance alone. They received either 320 mg ASP6294 or placebo. Study medicines were injected under the skin in the lower abdomen. Women received the injections at 4 week intervals.

This study took place at 26 clinics in several countries. 119 women were assigned to a treatment in the study. Out of these, 117 women took at least 1 dose of study medicine.

	Number of Women
Age Group	
Aged less than 65 years	92
Aged 65 years to less than 85 years	24
Aged 85 years and older	1
Clinic Location	
European Union Countries (*at the time of the study*)	93
Belgium	2
Czech Republic	7
Germany	6
Hungary	15
Latvia	27
The Netherlands	2
Poland	29
Spain	3
United Kingdom	2
Outside European Union	24
Russian Federation	24

What Were the Study Results?

Women with bladder pain syndrome with interstitial cystitis received either ASP6294 or placebo. The study looked how the patient's average daily pain changed from before the study medicine started until after 12 weeks.

Patients who took ASP6294 and patients who took placebo both had less pain at 12 weeks. The average change in daily pain from before the study medicine started until after 12 weeks was - 2.34 for the patients taking ASP6294. The change was - 2.13 for the patients taking placebo. A reduction in the pain score indicates the patient had less pain than they had at the beginning of the study. The difference between the 2 groups was - 0.20. Statistical testing showed that this difference was likely to be due to chance.

What Adverse Reactions did Patients Have?

A lot of research is needed to know whether a medicine causes a medical problem. So when new medicines are being studied researchers keep track of all medical problems that patients have while they are in the study. These medical problems are called "adverse events" and are recorded whether or not they might be caused by the treatment taken. An "adverse reaction"

is any medical problem or "adverse event" that is judged by the study doctor to be possibly caused by a medicine or treatment used in the study.

The table below shows the most common adverse reactions experienced by women who took at least 1 dose of study medicine in this study.

Adverse Reaction	**Placebo (out of 61 women)**	**ASP6294 (out of 56 women)**
Any adverse reaction	9 (14.8%)	12 (21.4%)
Joint pain	0	4 (7.1%)
Pain at the injection site	3 (4.9%)	0

An adverse reaction is considered "serious" when it is life-threatening, causes lasting problems or needs hospital care.

1 woman (1.6%, or 1 out of 61) experienced a serious adverse reaction in this study. The woman received placebo.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study. You can find this summary and more information about this study online at http://www.astellasclinicalstudyresults.com.

Please remember that researchers look at the results of many studies to find out how well medicines work and which adverse reactions they might cause. This summary only shows the results of this 1 study. Your doctor may help you understand more about the results of this study.

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