# ENGAGE-24:

## Evaluation of the Safety and Effectiveness of URG101 in Subjects With Interstitial Cystitis/Bladder Pain Syndrome

## Objective

To determine if the combination product, URG101 is safe and effective compared to its individual components, heparin sodium and lidocaine hydrochloride for the treatment of interstitial cystitis/bladder pain syndrome (IC/BPS).

## Study Design

**Allocation:** Randomized
**Endpoint Classification:** Safety/Efficacy Study
**Intervention Model:** Parallel Assignment
**Masking:** Double Blind (Subject, Caregiver, Investigator)
**Primary Purpose:** Treatment

Target Enrollment: **300** SUBJECTS

## Inclusion Criteria

1. Read, understand, and sign an informed consent form
2. Be male or female, ≥ 18 years of age
3. Have moderate-to-severe symptoms of bladder pain of bladder origin for at least 9 months prior to the study
4. Optional cystoscopy in association with your diagnosis of interstitial cystitis/bladder pain syndrome prior to or at time of screening
5. At screening complete a questionnaire that measures the symptoms associated with IC/BPS, based on pain, urgency, and frequency of urination (PUF questionnaire) with an overall eligible score
6. An eligible score for bladder pain is required on the Bladder Pain and Urgency 11-Point Numerical Rating Scales (NRS) on the day of treatment with the study drug
7. If female and currently taking hormone therapy, have been using a stable dose of hormone therapy for ≥ 3 months

**For more information, please contact us:**
**E-mail:** engage-24@cato.com

# Frequently Asked Questions

## What is the purpose of the study?

The purpose of this research study is to determine whether the study drug, URG101 is safe and effective in treating bladder pain in subjects with interstitial cystitis / bladder pain syndrome (IC/BPS). The study drug is a novel formulation of heparin and lidocaine aimed at delivering a safe and effective dose to anesthetize the bladder to reduce pain. The study is designed to test that the specially formulated product is better in relieving pain over 24 hours compared to lidocaine or heparin alone. The study drug is currently an investigational product, which means that the U.S. Food and Drug Administration has not evaluated for approval, the use of URG101 by the public.

## Who is eligible to participate in the study?

If you are diagnosed with interstitial cystitis/bladder pain syndrome, you must meet all of the following criteria to participate in the study:

1. Read, understand, and sign an informed consent form
2. Be male or female, ≥ 18 years of age
3. Have moderate-to-severe symptoms of bladder pain for at least 9 months prior to the study
4. Optional cystoscopy in association with your diagnosis of interstitial cystitis/bladder pain syndrome prior to or at time of screening
5. At screening complete a questionnaire that measures the symptoms associated with IC/BPS, based on pain, urgency, and frequency of urination (PUF questionnaire) with an overall eligible score
6. An eligible score for bladder pain is required on the Bladder Pain and Urgency 11-Point Numerical Rating Scales (NRS) on the day of treatment with the study drug
7. If female and currently taking hormone therapy, have been using a stable dose of hormone therapy for ≥ 3 months,

## If I am eligible to participate, which medication will I get?

Eligible subjects exhibiting moderate to severe symptoms of bladder pain associated with their diagnosis of interstitial cystitis/bladder pain syndrome who have signed informed consent, will be screened and provisionally enrolled in the ENGAGE-24 study.

On the day of study drug administration, you will be randomized so that you will have a 1 in 3 chance of receiving URG101 or lidocaine alone and a 1 in 6 chance of receiving placebo or heparin alone. You will receive a single administration of one of four intravesical treatments in a blinded fashion, based on random assignment:

1. URG101 (buffered lidocaine-heparin)
2. Placebo (buffer solution without lidocaine or heparin mixed in it)
3. Lidocaine Hydrochloride buffered alone
4. Heparin Sodium buffered alone

## What is the study duration?

Your active participation in this research study includes 24 hours of assessments after you receive the study drug treatment. Depending on the enrollment process time and the timing of the follow-up phone call (48 or 72 hours), your involvement in the study could last between 3 and 10 days.

## Will it cost me anything to participate in the study?

The sponsor of this study will pay for the procedures, tests, and treatments performed for research purposes. The expense of any procedures, tests, or treatments related to your normal medical care will be billed to you or your insurer in the regular way.

You will not be charged to participate in this research study.

## Do I get paid to participate in the study?

You may receive compensation for your travel expenses.

## What are the potential benefits?

It is hoped that this study drug and/or one of the other mixtures will relieve your condition, but it is not possible to guarantee that this study drug will help your condition. However, studies such as this give information that could help improve the treatment available for people with interstitial cystitis / bladder pain syndrome. It is possible that your symptoms may improve, stay the same, or get worse. If you still feel pain 24 hours after receiving the study medication, you may receive your physicians' standard of care treatment for IC/BPS.

## What are the risks?

The active ingredients in this study drug, lidocaine and heparin, have been approved individually by the FDA, but not for the treatment of interstitial cystitis / bladder pain syndrome. Because these two products, mixed with this specific formula have not gone through a formal approval process in the U.S., URG101 has not been approved by the FDA.

As with any study drug, there may be risks that are not expected or foreseen. Given the history and previous use of the active ingredients in this study drug, there are a number of known adverse events (a negative change in health or side effect that happens during a study) that you and your doctor should be aware of for this clinical study. (Please discuss with your doctor)

Lidocaine has possible discomforts or risks that include, but are not limited to, the following:

- Nervous System related events
- Heart related events
- Allergic reactions

Heparin has possible discomforts or risks that include, but are not limited to, the following:

- Excessive bleeding
- Hypersensitivity related events

## How am I protected from injury?

All forms of medical testing and treatment (both experimental and routine) involve some risk of injury. The study doctor will try to reduce the chances that you will experience discomfort or injury during this research study. In spite of all precautions, you may develop medical complications from participating in this research study.

In the case of research-related side effects or injury, medical care will be provided by your doctor or you will be referred for appropriate medical care. Although no funds have been set aside to compensate you in the event of injury or illness related to the study drug or procedures, you do not waive your legal right to such compensation by signing the study consent form to participate in this study.

## Can I withdraw from the study?

Your participation in this research study is voluntary. You may refuse to participate or stop your participation at any time without penalty. If you choose not to participate or stop participation, the quality of your health care will not be affected. The study doctor has the right to stop your

participation in the research study at any time. Your study doctor could decide to stop your participation because you have an unexpected reaction, because you have failed to follow instructions given to you by the study staff, or because the entire study has been stopped. If you should withdraw from the research study or your study doctor stops your participation, you will be asked to come to the clinic for a final visit for a general safety evaluation; however, this may not be required.

## Who will have access to my information and results?

Your medical records are protected by privacy standards. However, the purpose of this research study is to produce medical record information about you and your medical progress during the research study to help determine the safety and effectiveness of URG101. The collected information will be placed in your medical records. This may include information that can be used to identify you (such as your name, address, date of birth, or date of admission to the hospital). This could include information about HIV and genetic testing, or treatment for drug or alcohol abuse or mental health problems.

The study doctor, the sponsor or persons working on behalf of the sponsor, the United States Food and Drug Administration (FDA), other U.S. regulatory authorities, the Independent Review Board (IRB), and possibly governmental agencies of other countries will be able to inspect and copy confidential study-specific records which identify you by name. Therefore, absolute confidentiality cannot be guaranteed. Records of your participation in this study will be held confidential to the extent permitted by the applicable laws and regulations, and consistent with the Health Insurance Portability and Accountability Act (“HIPAA”) Authorization that you will be asked to sign. For more information on how your personal health information will be disclosed, please refer to the Health Insurance Portability and Accountability Act (“HIPAA”) Authorization.

## I want to participate in the study, who should I contact?

Ask your doctor if you qualify to participate in this study.

If you feel like you qualify for this study, you may contact us:

**E-mail:** engage-24@cato.com

Once your location is determined, you will be informed of the possible locations where the study is currently taking place.