Samsca drug use-results survey (ADPKD)

Study Protocol

NCT Number: NCT02847624

Otsuka Pharmaceutical Co., Ltd.

Protocol Creation Date: February 21, 2014, Version 1.0
Date of the latest version: September 2, 2021, Version 8.0

Objective of survey	To determine the safety and efficacy of Samsca used in routine clinical settings for suppression of ADPKD progression.
Survey methods	<All patients survey> All patients treated with Samsca must be registered in the survey until "CONDITIONS FOR APPROVAL" is removed. CRFs must be collected from all patients until the total number of patients registered in the survey reaches the planned number. Observation period: From start of Samsca administration through the end of survey In case of finish or discontinue of Samsca administration, collecting information of kidney volume, changes in renal function and hepatic dysfunction (outcome: not recovered) through the end of survey (8 years).
Survey period	8 years (March 24,2014 ~ March 23,2022)
Planned number of patients to be surveyed	1,600 Depending on the onset of significant drug-induced liver injury post-marketing, the planned number of patients will be re-evaluated and necessity of additional safety measures will be also assessed. In addition, if there is a substantial change in expected number of patients with long-term treatment (≥4 years), the planned number of patients will be re-evaluated so as to collect at least 800 patients with long-term treatment.
Target patient population of survey	All patients treated with Samsca after March 24, 2014 for the indication of "suppression of ADPKD progression with a rapid kidney volume increase in patients with increased kidney volume"
Major items to be surveyed	**CRFs of drug use-results survey (for treatment period)** 1) Patient information Patient initials (last name, first name), patient ID, sex, any breast feeding, any pregnancy, date of birth (age), date of informed consent received (in written form) 2) Patient demographics Indication for use, time of ADPKD diagnosis, diagnosis of ADPKD (severity of ADPKD, family history, number of cysts, complications (especially, hepatic cyst, pancreatic cyst, cerebral aneurysm, urinary calculus, any complication of cyst infection), past history, predisposition to hypersensitivity, history of drug adverse reactions 3) Administration status of Samsca Dosage form, time and dose, route of administration, period of administration (date started, date stopped), 4) Usage status of Samsca Ongoing, completed, discontinued (reason for discontinuation → adverse events, insufficient efficacy, changing hospital, other), no visit 5) Changes in kidney volume In-patient/out-patient status, total kidney volume (measured or not, method of measurement), change of kidney volume 6) Laboratory values and electrocardiography (1) Physical examination

Height, weight, body temperature, ECG finding

(3) Blood chemistry tests

AST (GOT), ALT (GPT), e-GFR

(4) Urinalysis

Urinary protein, urine sugar, bilirubin, urobilinogen, urine albumin, urine osmolality

7) Adverse events after start of Samsca administration

Specific adverse events, onset date, seriousness, reason for seriousness (if serious), date of outcome assessment, outcome, causal relationship to Samsca, other causative factors than Samsca, corrective treatment for adverse event

8) Condition around the event onset

Condition around the event onset, comments on the adverse events

CRFs of drug use-results survey (for follow-up period)

1) Patient information

Patient initials (last name, first name), patient ID, sex, any breast feeding, any pregnancy, date of birth (age), date of informed consent received (in written form)

2) Changes in kidney volume

In-patient/out-patient status, total kidney volume (measured or not, method of measurement), change of kidney volume,

3) Laboratory values

(1) Physical examination

Height, weight

(2) Blood chemistry tests

AST (GOT), ALT (GPT), e-GFR

4) Follow-up investigation of adverse events that remained unresolved or were of unknown status during the treatment period.

Specific adverse events, onset date, seriousness, reason for seriousness (if serious), date of outcome assessment, outcome, causal relationship to Samsca, other causative factors than Samsca, corrective treatment for adverse event

5) Condition around the event onset and comments on the adverse events

Items to be analyzed	**CRFs of drug use-results survey (for treatment period)** 1) Items related to constitution of patients Number of patients enrolled, number of patients withdrawn, number of CRFs collected, number of patients excluded, number of patients analyzed for safety, number of patients analyzed for efficacy 2) Items related to efficacy Changes in kidney volume data, changes in renal function test data (e-GFR) 3) Items related to safety (1) Safety in elderly patients (2) Safety of Samsca in advanced ADPKD patients (creatinine clearance <60 mL/min) (3) Adverse events of special interest (hepatic dysfunction, increases in transaminases (AST or ALT)) (4) Items to be closely monitored: hepatic dysfunction (5) Safety of Samsca in ADPKD patients after long-term treatment **CRFs of drug use-results survey (for follow-up period)** 1) Changes in kidney volume, changes in renal function test data (e-GFR)