ClinicalTrials.gov Protocol Registration and Results System (PRS) Receipt
Release Date: September 26, 2023

ClinicalTrials.gov ID: NCT05098509

Study Identification

Unique Protocol ID:	SCOUT-015
Brief Title:	A Study to Assess RAD011 (Cannabidiol Oral Solution) for the Treatment of Participants With Prader-Willi Syndrome
Official Title:	A Phase 2/3, Randomized, Double-Blind, Placebo-Controlled Study of RAD011 (Cannabidiol Oral Solution) for the Treatment of Patients With Prader- Willi Syndrome
Secondary IDs:	

Study Status

Record Verification:	September 2023
Overall Status:	Terminated [Due to a change in corporate priorities, this study was voluntarily terminated by the Sponsor for reasons other than safety]
Study Start:	April 13, 2022 [Actual]
Primary Completion:	October 6, 2022 [Actual]
Study Completion:	October 31, 2022 [Actual]

Sponsor/Collaborators

Sponsor:	Radius Pharmaceuticals, Inc.
Responsible Party:	Sponsor
Collaborators:	

Oversight

U.S. FDA-regulated Drug:	Yes

U.S. FDA-regulated Device:	No
U.S. FDA IND/IDE:	Yes
IND/IDE Information:	FDA Center: CDER IND/IDE Number: 136,374 Serial Number: Has Expanded Access: No
Human Subjects Review:	Board Status: Approved Board Name: Advarra Board Affiliation: Central IRB Phone: 410-884-2900 Email: adviser@advarra.com Address: 6100 Merriweather Dr., Suite 600 Columbia, MD 21044
Data Monitoring:	Yes
FDA Regulated Intervention:	No

Study Description

Brief Summary:	This was a study investigating RAD011 in participants diagnosed with Prader-Willi Syndrome (PWS). The primary objective of the Phase 2 part of this study was to assess the safety and tolerability of multiple dose levels of RAD011 in order to select 1 or 2 dose level(s) for further evaluation in the Phase 3 part of the study. In Phase 3, the primary objective was to assess the effect of RAD011 on hyperphagia-related behavior in participants with PWS.
Detailed Description:	

Conditions

Conditions:	Prader-Willi Syndrome
Keywords:	

Study Design

Study Type:	Interventional
Primary Purpose:	Treatment
Study Phase:	Phase 2/Phase 3
Interventional Study Model:	Parallel Assignment

Number of Arms: 4

Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor)

Allocation: Randomized

Enrollment: 4 [Actual]

Arms and Interventions

Arms	Assigned Interventions
Active Comparator: RAD011 40 milligrams per kilogram (mg/kg) Participants were administered 40 mg/kg of RAD011 orally daily with food.	Drug: RAD011 Cannabidiol Oral Solution (containing synthetic cannabidiol)
Active Comparator: RAD011 20 mg/kg Participants were administered 20 mg/kg of RAD011 orally daily with food.	Drug: RAD011 Cannabidiol Oral Solution (containing synthetic cannabidiol)
Active Comparator: RAD011 10 mg/kg Participants were administered 10 mg/kg of RAD011 orally daily with food.	Drug: RAD011 Cannabidiol Oral Solution (containing synthetic cannabidiol)
Placebo Comparator: Placebo Participants were administered a placebo matching to RAD011, orally daily with food.	Drug: Placebo Matching Placebo for RAD011

Outcome Measures

[See Results Section.]

Eligibility

Minimum Age: 8 Years

Maximum Age: 65 Years

Sex: All

Gender Based: No

Accepts Healthy Volunteers: No

Criteria: Inclusion Criteria:

- Males and females between 8 and 65 years of age (inclusive) at Screening.
- Genetically confirmed diagnosis of PWS. Documentation of genetically confirmed diagnosis of PWS is acceptable.
- The same caregiver is available to complete the questionnaire throughout the duration of the study.
- After completion of the Tolerability period, participants will have a mean Hyperphagia Questionnaire for Clinical Trials (HQ-CT) score ≥13 and a decrease of HQ-CT score no more than 7 during Tolerability (run-in) period.
- If receiving growth hormone, psychotropic therapy, metabolic treatments that could affect appetite (including metformin), and other treatment including thyroid hormone, must be on the same medication and dose for at least 90 days prior to consent/assent

Exclusion Criteria:

- Known use of cannabis or cannabinoid containing products (including topical products) within 90 days prior to consent/ assent.
- Use of prescription or over-the-counter weight loss agents within 90 days prior to consent/assent.
- Implementation of new food or environmental restrictions within 90 days of consent/ assent.
- If living in a group home, participant spends less than 25 waking hours with their caregiver per week.
- Uncontrolled chronic conditions (diabetes, sleep apnea, etc.) as determined by the Investigator.

Contacts/Locations

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IPDSharing

Plan to Share IPD:

References

Citations:

Links:

Available IPD/Information:

Documents

Statistical Analysis Plan
Document Date: March 10, 2022
Uploaded: 08/16/2023 13:06

Study Protocol
Document Date: March 10, 2022
Uploaded: 08/16/2023 13:07

Study Results

Participant Flow

Pre-assignment Details	In this study, 4 participants were randomized and received at least 1 dose of study drug. No participants were randomized to the placebo group. The 4 participants were randomized in Phase 2 of the study and due to premature termination of the study, phase 3 was not conducted. None of the enrolled participants completed the study due to the study being terminated prematurely.

Reporting Groups

	Description
RAD011 10 Milligrams Per Kilogram (mg/kg)	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.
Placebo	Participants were administered a placebo matching to RAD011, orally daily with food.

Overall Study

	RAD011 10 Milligrams Per Kilogram (mg/kg)	RAD011 20 mg/kg	RAD011 40 mg/kg	Placebo
Started	1	1	2	0
Received 1 Dose of Study Drug	1	1	2	0
Completed	0	0	0	0
Not Completed	1	1	2	0
Withdrawal by Subject	0	0	1	0

	RAD011 10 Milligrams Per Kilogram (mg/kg)	RAD011 20 mg/kg	RAD011 40 mg/kg	Placebo
Early study termination	1	1	1	0

Baseline Characteristics

Baseline Analysis Population Description

The Intent to Treat (ITT) Population included all randomized participants who received at least 1 dose of study drug. Due to the small sample size, data was not reported for confidentiality reasons.

Reporting Groups

	Description
RAD011 10 mg/kg	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.

Baseline Measures

		RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg	Total
Overall Number of Participants		1	1	2	4
Age, Continuous Median (Full Range) Unit of measure: years	Number Analyzed	1 participants	1 participants	2 participants	4 participants
		NA (NA to NA) [1]	NA (NA to NA) [1]	NA (NA to NA) [1]	NA (NA to NA) [1]
		[1] Due to the small sample size for this baseline characteristic, data was not reported for confidentiality reasons.			
Sex: Female, Male [1] Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	0 participants	0 participants	0 participants	0 participants
	Female	0	0	0	0
	Male	0	0	0	0
		[1] Measure Analysis Population Description: Due to the small sample size for this baseline characteristic, data was not reported for confidentiality reasons.			

		RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg	Total
Ethnicity (NIH/OMB) [1] Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	0 participants	0 participants	0 participants	0 participants
	Hispanic or Latino	0	0	0	0
	Not Hispanic or Latino	0	0	0	0
	Unknown or Not Reported	0	0	0	0
		[1] Measure Analysis Population Description: Due to the small sample size for this baseline characteristic, data was not reported for confidentiality reasons.			
Race (NIH/OMB) [1] Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	0 participants	0 participants	0 participants	0 participants
	American Indian or Alaska Native	0	0	0	0
	Asian	0	0	0	0
	Native Hawaiian or Other Pacific Islander	0	0	0	0
	Black or African American	0	0	0	0
	White	0	0	0	0
	More than one race	0	0	0	0
	Unknown or Not Reported	0	0	0	0
		[1] Measure Analysis Population Description: Due to the small sample size for this baseline characteristic, data was not reported for confidentiality reasons.			

Outcome Measures

1. Primary Outcome Measure:

Measure Title	Change From Baseline to Week 34 in the Hyperphagia Questionnaire for Clinical Trials (HQ-CT) Questionnaire
Measure Description	The HQ-CT measures hyperphagia by Prader-Willi syndrome (PWS)-specialized clinicians. The HQ-CT generates a score ranging from 0 to 36, where a higher score represents more severe abnormal food related behaviors. The change from baseline was calculated from two time points as the value at the later time point minus the value at the earlier time point.
Time Frame	Baseline, Week 34

Analysis Population Description

Due to the premature termination of the study, no efficacy analyses were conducted. Therefore, no data was collected for this outcome measure.

Reporting Groups

	Description
RAD011 10 mg/kg	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.

Measured Values

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
Overall Number of Participants Analyzed	0	0	0

No data displayed because Outcome Measure has zero total participants analyzed.

2. Secondary Outcome Measure:

Measure Title	Change From Baseline to Week 34 in Prader-Willi Syndrome (PWS)- Associated Irritability Using the Aberrant Behavior Checklist (ABC) Questionnaire - Irritability Subscale (ABC-I)
Measure Description	The ABC questionnaire is an informant-rated questionnaire assessing severity of behavioral symptoms. The Irritability subscale of the ABC covers symptoms such as agitation, aggression, meltdowns, and self-harm. The ABC-I contains 15 items and each item is scored as 0 (never a problem), 1 (slight problem), 2 (moderately serious problem), or 3 (severe problem). The total score is the sum of individual items scores which ranges from 0 (no problem) to 45 (severe problem), with higher score indicating more severe condition.
Time Frame	Baseline, Week 34

Analysis Population Description

Due to the premature termination of the study, no efficacy analyses were conducted. Therefore, no data was collected for this outcome measure.

Reporting Groups

	Description
RAD011 10 mg/kg	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.

Measured Values

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
Overall Number of Participants Analyzed	0	0	0

No data displayed because Outcome Measure has zero total participants analyzed.

3. Secondary Outcome Measure:

Measure Title	Change From Baseline to Week 34 in Hyperphagia as Measured by the Clinician Global Impression of Change (CGI-C)
Measure Description	The CGI-C of hyperphagia is a single-item, clinician-rated measure, assessing the clinician's impression about changes in the patient's hyperphagia condition since the start of taking the study medication at the initiation of the Tolerability Period. The CGI-C of hyperphagia utilizes a 5-point response scale: 1=Much better; 2=A little better; 3=No change; 4=A little worse; 5=Much worse. Higher scores mean a worse outcome.
Time Frame	Baseline, Week 34

Analysis Population Description

Due to the premature termination of the study, no efficacy analyses were conducted. Therefore, no data was collected for this outcome measure.

Reporting Groups

	Description
RAD011 10 mg/kg	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.

Measured Values

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
Overall Number of Participants Analyzed	0	0	0

No data displayed because Outcome Measure has zero total participants analyzed.

4. Secondary Outcome Measure:

Measure Title	Change From Baseline to Week 34 in Hyperphagia as Measured by the Clinician Global Impression of Severity (CGI-S)
Measure Description	The CGI-S of hyperphagia is a single-item, clinician-rated measure, assessing the clinician's impression of the severity of a patient's hyperphagia condition. The CGI-S of hyperphagia utilizes a 5-point response scale: 1=None; 2=Mild; 3=Moderate; 4=Severe; 5=Very Severe. Higher scores mean a worse outcome.
Time Frame	Baseline, Week 34

Analysis Population Description

Due to the premature termination of the study, no efficacy analyses were conducted. Therefore, no data was collected for this outcome measure.

Reporting Groups

	Description
RAD011 10 mg/kg	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.

Measured Values

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
Overall Number of Participants Analyzed	0	0	0

No data displayed because Outcome Measure has zero total participants analyzed.

Reported Adverse Events

Time Frame	Day 1 (after dosing) up to 68 days (maximum duration from treatment to early termination)
Adverse Event Reporting Description	Safety population included all participants who received at least 1 dose of study medication.

Reporting Groups

	Description
RAD011 10 mg/kg	Participants were administered 10 mg/kg of RAD011 orally daily with food.
RAD011 20 mg/kg	Participants were administered 20 mg/kg of RAD011 orally daily with food.
RAD011 40 mg/kg	Participants were administered 40 mg/kg of RAD011 orally daily with food.

All-Cause Mortality

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
	Affected/At Risk (%)	Affected/At Risk (%)	Affected/At Risk (%)
Total All-Cause Mortality	0/1 (0%)	0/1 (0%)	0/2 (0%)

Serious Adverse Events

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
	Affected/At Risk (%)	Affected/At Risk (%)	Affected/At Risk (%)
Total	0/1 (0%)	0/1 (0%)	0/2 (0%)

Other Adverse Events

Frequency Threshold Above Which Other Adverse Events are Reported: 0%

	RAD011 10 mg/kg	RAD011 20 mg/kg	RAD011 40 mg/kg
	Affected/At Risk (%)	Affected/At Risk (%)	Affected/At Risk (%)
Total	1/1 (100%)	1/1 (100%)	2/2 (100%)
Ear and labyrinth disorders			
Ear discomfort †	0/1 (0%)	0/1 (0%)	1/2 (50%)
Gastrointestinal disorders			
Diarrhea †	0/1 (0%)	0/1 (0%)	2/2 (100%)
General disorders			
Fatigue †	0/1 (0%)	0/1 (0%)	1/2 (50%)
Nervous system disorders			
Headache †	0/1 (0%)	0/1 (0%)	1/2 (50%)
Lethargy †	0/1 (0%)	1/1 (100%)	0/2 (0%)
Psychiatric disorders			
Irritability †	0/1 (0%)	0/1 (0%)	1/2 (50%)
Skin and subcutaneous tissue disorders			
Dermatitis †	1/1 (100%)	0/1 (0%)	0/2 (0%)

† Indicates events were collected by systematic assessment.

Limitations and Caveats

The study has been voluntarily terminated by the sponsor due to change in corporate priorities, for reasons other than safety. Due to early termination of study, no efficacy analyses were done.

More Information

Certain Agreements:

Principal Investigators are NOT employed by the organization sponsoring the study.

There IS an agreement between the Principal Investigator and the Sponsor (or its agents) that restricts the PI's rights to discuss or publish trial results after the trial is completed.

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