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Clinical Trial Results Database

Sponsor

Novartis

Generic Drug Name

AFQ056

Therapeutic Area of Trial

Fragile X syndrome

Approved Indication

None

Protocol Number

CAFQ056B2154

Title

Sequential, two-period study to assess the pharmacokinetics, safety & tolerability of single and multiple oral doses of AFQ056 in patients with Fragile X syndrome aged 5-11 years (Cohort 1) and 3-4 years (Cohort 2)

Study phase

Phase I

Study Start/End Dates

23-Mar-2012 (first patient first visit) to 16-Oct-2013 (last patient last visit)

Study Design/Methodology

This was a sequential, open-label; multi-centered study that assessed the pharmacokinetics of AFQ056 in pediatric patients with Fragile X syndrome (5-11 years old in cohort 1 and 3-4 years old in cohort 2). In addition, the safety and tolerability of single and multiple oral AFQ056 doses in this patient population was evaluated.

Cohort 2 commenced only after an interim analysis of the PK and safety/tolerability data was completed for cohort 1. Patients in cohort 2 were exposed to AFQ056, only after it was confirmed that the selected dose is safe and well tolerated based on the data obtained from cohort 1. In period 1, single dose of AFQ056 15 mg was administered. In period 2, subjects of cohort 1 and cohort 2 received oral doses of either 1.25 mg/kg or 50 mg or 100 mg b.i.d. AFQ056 over seven or eight days, respectively.

Centers

Two centers in USA and one center in Spain.

Publication

None

Objectives

Primary objective

To assess the pharmacokinetics of single and multiple oral AFQ056 doses in patients with FXS aged 5-11 years (cohort 1) and 3-4 years if included in the study (cohort 2)

Secondary objective

To evaluate the safety and tolerability of single and multiple oral AFQ056 in patients with FXS aged 5-11 years (cohort 1) and 3-4 years if included in the study (cohort 2)

Test Product, Dose, and Mode of Administration

The investigational drug (AFQ056) was prepared by Novartis and provided to the Investigator as open label medication in a form of a powder for an oral suspension, resulting after reconstitution with water in a 10 mg/mL oral suspension. Similar formulation had previously been administered in a single-dose relative bioavailability study in healthy adults (CAFQ056A2166).. In situ reconstitution of suspension was done on site by an authorized person. The study drug was orally administered to the patients in the syringe.

Statistical Methods

Descriptive statistics was provided for primary and secondary PK parameters. In addition descriptive statistics was provided for dose normalized primary pharmacokinetic parameters of AFQ056. Descriptive statistics of pharmacokinetic parameters included mean, standard deviation, and coefficient of variation, minimum and maximum. When a geometric mean was presented it was stated as such. Since Tmax was generally evaluated by a nonparametric method, median values and ranges were given for this parameter.

For the pharmacokinetic parameters of primary interest, the geometric mean of dose normalized values and 90% confidence intervals were calculated for the data by treatment (different doses of AFQ056, single and multiple doses as per availability of the data). The calculations were done on the log-transformed pharmacokinetic parameters and the results were back-transformed to original scale afterwards.

All the above analysis was provided for cohort 1 and cohort 2 separately according to availability of the data.

Interim Analysis: An interim analysis was performed after completion of cohort 1.

The sponsor and investigators analyzed and discussed all available pharmacokinetic as well as safety and tolerability data. The decision was taken to continue the study with cohort 2 (patients aged 3 to 4 years) was based primarily on the safety and tolerability of AFQ056 in cohort 1.

Study Population: Inclusion/Exclusion Criteria and Demographics

Key Inclusion Criteria

Patients eligible for inclusion in this study had to fulfill all of the following criteria:

- Male or female patients with Fragile X syndrome, aged between 5 and 11 years inclusive (for cohort 1) and aged between 3 and 4 years inclusive (for cohort 2).
- Genetically confirmed diagnosis of Fragile X syndrome (>200 CGG repeats in the fmr1 gene (historic data acceptable if available).
- Body weight must be ≥ the age- and gender-specific 10th percentile
- Body mass index must be ≥ the age- and gender-specific 10th percentile

Key Exclusion criteria:

Patients fulfilling any of the following criteria were not eligible for inclusion in this study:

- History or presence of any clinically significant disease of any major system organ class, within the past 2 years prior to screening including but not limited to psychiatric, neurological, cardiovascular, endocrine, metabolic, renal, or gastrointestinal disorders. This does not include typical features of Fragile X syndrome such as psychological symptoms, dysmorphia or history of epileptic seizures
- Smokers (use of tobacco products in the previous 3 months).
- Any abnormal laboratory values at screening or first baseline that are in the opinion of

the investigator clinically significant and may jeopardize the safety of the study subject

- Use of (or use within at least 5 half-lives before dosing) concomitant medications that are strong/moderate inhibitors or inducers of CYP1A1/2, CYP2C9/19 or CYP3A4
- Use of (or use within at least 5 half-lives before dosing) concomitant medications that are strong/moderate inhibitors or inducers of CYP1A1/2, CYP2C9/19 or CYP3A4

Participant Flow

Patient disposition - n(percent) of patients (Safety analysis set)

	Cohort1 N=12 n (%)	Cohort2 N=9 n (%)	All Patients N=21 n (%)
Patients			
Completed	12 (100)	9 (100)	21 (100)

Baseline Characteristics

Demographic summary by Cohort (Safety analysis set)

		Cohort 1			Cohort 2	All
		(5-7yrs) N=6	(8-11yrs) N=6	(All patients) N=12	(3-4yrs) N=9	Patients N=21
Age (years)	Mean (SD)	5.8 (0.98)	9.5 (1.05)	7.7 (2.15)	3.9 (0.33)	6.0 (2.50)
	Range	5 - 7	8 - 11	5 - 11	3 - 4	3 - 11
Height (cm)	Mean (SD)	118.8 (5.98)	141.3 (4.50)	130.1 (12.79)	107.8 (6.16)	120.5 (15.27)
	Range	111 - 128	137 - 149	111 - 149	93 - 113	93 - 149
Weight (kg)	Mean (SD)	24.22 (9.526)	40.47 (10.720)	32.34 (12.865)	19.40 (2.621)	26.80 (11.698)
	Range	18.6 - 43.2	28.1 - 55.0	18.6 - 55.0	15.0 - 23.0	15.0 - 55.0
BMI (kg/m^2)	Mean (SD)	16.80 (4.754)	20.09 (4.425)	18.44 (4.704)	16.69 (1.594)	17.69 (3.739)
	Range	13.8 - 26.4	14.8 - 25.1	13.8 - 26.4	13.6 - 18.3	13.6 - 26.4
Sex - n(%)	Male	6 (100)	5 (83.3)	11 (91.7)	8 (88.9)	19 (90.5)
	Female	0 (0.0)	1 (16.7)	1 (8.3)	1 (11.1)	2 (9.5)
Race - n(%)	Caucasian	5 (83.3)	4 (66.7)	9 (75.0)	8 (88.9)	17 (81.0)
	Black	0 (0.0)	0 (0.0)	0 (0.0)	1 (11.1)	1 (4.8)
	Asian	1 (16.7)	1 (16.7)	2 (16.7)	0 (0.0)	2 (9.5)
	Other	0 (0.0)	1 (16.7)	1 (8.3)	0 (0.0)	1 (4.8)
Ethnicity - n(%)	Hispanic/Latino	0 (0.0)	1 (16.7)	1 (8.3)	0 (0.0)	1 (4.8)
	Chinese	1 (16.7)	1 (16.7)	2 (16.7)	0 (0.0)	2 (9.5)
	Other	5 (83.3)	4 (66.7)	9 (75.0)	9 (100)	18 (85.7)

Summary of Efficacy

Primary Outcome Results

Cohort 1

Summary statistics for pharmacokinetic variables of primary interest after single oral administration of AFQ056 15 mg in patients with Fragile X syndrome aged 5-11 years

Treatment		Cmax (ng/mL)	AUC0-12h (ng.h/mL)	AUClast (ng.h/mL)	AUCinf (ng.h/mL)
AFQ056 15 mg	n	12	11	12	8
	Mean (SD)	52.0 (36.8)	190 (101)	230 (110)	264 (134)
	CV% mean	70.7	52.9	47.6	50.7
	Geo mean	43.1	168	207	232
	CV% geo-mean	68.4	58.1	52.2	63.1

Summary statistics for pharmacokinetic parameters after multiple oral doses of AFQ056 on Day 7 in patients with Fragile X syndrome aged 5-11 years

Treatment	Statistic	Cmax (ng/mL)	AUC0-12h (hr*ng/mL)	AUClast (hr*ng/mL)	Tmax (hr)
AFQ056 20 mg b.i.d.	n	1	-	1	1
	Mean (SD)	196 (-)	-	510 (-)	2.00 (-)
	CV% mean	-	-	-	-
	Geo-mean	196	-	510	2.00
	CV% geo-mean	-	-	-	-
	Median	196	-	510	2.00
	[Min; Max]	[196;196]	-	[510;510]	[2.00;2.00]
AFQ056 25 mg b.i.d.	n	1	1	1	1
	Mean (SD)	113 (-)	461 (-)	419 (-)	0.500 (-)
	CV% mean	-	-	-	-
	Geo-mean	113	461	419	0.500
	CV% geo-mean	-	-	-	-
	Median	113	461	419	0.500
	[Min; Max]	[113;113]	[461;461]	[419;419]	[0.500;0.500]
AFQ056 50 mg b.i.d.	n	3	1	3	3
	Mean (SD)	229 (101)	585 (-)	913 (451)	1.57 (0.823)
	CV% mean	43.8	-	49.4	52.6
	Geo-mean	215	585	843	1.37
	CV% geo-mean	44.9	-	51.8	78.2
	Median	201	585	790	2.00
	[Min; Max]	[146;341]	[585;585]	[536;1410]	[0.617;2.08]
AFQO56 60 mg b.i.d.	n	1	-	1	1
	Mean (SD)	234 (-)	-	1210 (-)	2.00 (-)
	CV% mean	-	-	-	-
	Geo-mean	234	-	1210	2.00

	CV% geo-mean	-	-	-	-
	Median	234	-	1210	2.00
	[Min; Max]	[234;234]	-	[1210;1210]	[2.00;2.00]
AFQ056 100 mg b.i.d.	n	6	3	6	6
	Mean (SD)	286 (107)	1210 (114)	1110 (375)	1.03 (0.809)
	CV% mean	37.5	9.4	33.9	78.3
	Geo-mean	272	1210	1050	0.815
	CV% geo-mean	35.0	9.4	36.7	83.5
	Median	271	1200	1060	0.542
	[Min; Max]	[189;484]	[1100;1330]	[608;1660]	[0.467;2.15]

Geometric means and 90 percent confidence interval for dose-normalized pharmacokietic parameters of AFQ056 in plasma in patients with Fragile X syndrome aged 5-11 years

Cohort	Period	PK parameter [unit]	N	Geometric mean	90% CI for geometric mean
1	1	Cmax,ss/dose [ng/mL/mg]	12	2.88	[2.09 , 3.97]
		AUC0-12h,ss/dose [h*ng/mL/mg]	11	11.2	[8.32 , 15]
	2	Cmax,ss/dose [ng/mL/mg]	12	3.65	[2.84 , 4.69]
		AUC0-12h,ss/dose [h*ng/mL/mg]	5	13.1	[10.8 , 15.9]

Cohort 2

Summary statistics for pharmacokinetic variables of primary interest after single oral administration of AFQ056 15 mg in patients with Fragile X syndrome children aged between 3-4 years

Treatment		Cmax (ng/mL)	AUC0-12h (ng.h/mL)	AUClast (ng.h/mL)	AUCinf (ng.h/mL)
AFQ056 15 mg	n	9	9	9	8
	Mean (SD)	74.4 (27.4)	260 (104)	299 (130)	335 (128)
	CV% mean	36.9	40.0	43.5	38.2
	Geo mean	70.0	246	277	316
	CV% geo-mean	38.4	35.4	41.8	36.2

Summary statistics for pharmacokinetic parameters after multiple oral administration of AFQ056 on Day 8 in patients with Fragile X syndrome aged between 3-4 years

Treatment	Statistic	Cmax (ng/mL)	AUC0-12h (hr*ng/mL)	AUClast (hr*ng/mL)	Tmax (hr)
AFQ056 15 mg b.i.d.	n	1	-	1	1
	Mean (SD)	177	-	457	0.500
	CV% mean	-	-	-	-
	Geo-mean	177	-	457	0.500
	CV% geo-mean	-	-	-	-

	Median	177	-	457	0.500
	[Min; Max]	[177;177]	-	[457;457]	[0.500;0.500]
AFQ056 20 mg b.i.d.	n	1	-	1	1
	Mean (SD)	75.4	-	293	2.15
	CV% mean	-	-	-	
	Geo-mean	75.4	-	293	2.15
	CV% geo-mean	-	-	-	-
	Median	75.4	-	293	2.15
	[Min; Max]	[75.4;75.4]	-	[293;293]	[2.15;2.15]
AFQ056 25 mg b.i.d.	n	2	2	2	2
	Mean (SD)	149 (50.2)	689 (435)	541 (315)	0.684 (0.260)
	CV% mean	33.8	63.1	58.2	38.0
	Geo-mean	144	617	493	0.658
	CV% geo-mean	35.5	76.5	68.2	40.4
	Median	149	689	541	0.684
	[Min; Max]	[113;184]	[381;997]	[318;763]	[0.500;0.867]
AFQ056 50 mg b.i.d.	n	4	2	4	4
	Mean (SD)	179 (102)	768 (14.2)	726 (176)	1.32 (0.879)
	CV% mean	57.0	1.9	24.3	66.8
	Geo-mean	161	768	711	1.07
	CV% geo-mean	54.4	1.9	23.4	89.3
	Median	144	768	685	1.31
	[Min; Max]	[99.4;328]	[758;778]	[559;975]	[0.500;2.15]
AFQ056 100 mg b.i.d.	n	1	-	1	1
	Mean (SD)	96.0	-	267	2.00
	CV% mean	-	-	-	-
	Geo-mean	96.0	-	267	2.00
	CV% geo-mean	-	-	-	-
	Median	96.0	-	267	2.00
	[Min; Max]	[96.0;96.0]	-	[267;267]	[2.00;2.00]

Geometric means and 90 percent confidence interval for dose-normalized pharmacokinetic parameters of AFQ056 in plasma in patients with Fragile X syndrome aged 3-4 years

Cohort	Period	PK parameter [unit]	N	Geometric mean	90% CI for geometric mean
1	1	Cmax,ss/dose [ng/mL/mg]	9	4.67	[3.71 , 5.88]
		AUC0-12h,ss/dose [h*ng/mL/mg]	9	16.4	[13.3 , 20.3]
	2	Cmax,ss/dose [ng/mL/mg]	9	3.77	[2.36 , 6.01]
		AUC0-12h,ss/dose [h*ng/mL/mg]	4	19.5	[11.1 , 34.2]

Statistical analysis of AFQ056 treatment between two cohorts

Geometric means and 90 percent confidence interval for pharmacokinetic parameters of AFQ056 in plasma after single oral administration of AFQ056 at 15 mg in Cohort 1 (Fragile X syndrome aged 5-11 years) and Cohort 2 (Fragile X syndrome aged 3-4 years)

Cohort	Treatment	PK parameter [unit]	N	Geometric mean	90% CI for geometric mean

1	AFQ056 15 mg	Cmax [ng/mL]	12	43.1	[31.3 , 59.5]
		AUC0-12h [hr*ng/mL]	11	168	[125 , 225]
2	AFQ056 15 mg	Cmax,ss [ng/mL]	9	70	[55.7 , 88.1]
		AUC0-12h,ss [hr*ng/mL]	9	246	[199 , 304]

Geometric means and 90 percent confidence interval for dose-normalized pharmacokinetic parameters of AFQ056 in plasma after multiple doses of AFQ056 on Day 7/Day 8 in Cohort 1 (Fragile X syndrome aged 5-11 years) and Cohort 2 (Fragile X syndrome aged 3-4 years)

Cohort	Period	PK parameter [unit]	N	Geometric mean	90% CI for geometric mean
1	1	Cmax/dose [ng/mL/mg]	12	2.88	[2.09 , 3.97]
		AUC0-12h/dose [h*ng/mL/mg]	11	11.2	[8.32 , 15]
	2	Cmax,ss/dose [ng/mL/mg]	12	3.65	[2.84 , 4.69]
		AUC0-12h,ss/dose [h*ng/mL/mg]	5	13.1	[10.8 , 15.9]
2	1	Cmax/dose [ng/mL/mg]	9	4.67	[3.71 , 5.88]
		AUC0-12h/dose [h*ng/mL/mg]	9	16.4	[13.3 , 20.3]
	2	Cmax,ss/dose [ng/mL/mg]	9	3.77	[2.36 , 6.01]
		AUC0-12h,ss/dose [h*ng/mL/mg]	4	19.5	[11.1 , 34.2]

Secondary Outcome Results

Cohort 1

Summary statistics for pharmacokinetic variables of secondary interest after single oral administration of 15 mg of AFQ056 Fragile X syndrome children aged 5-11 years

Treatment		Tmax (h)	CL/F (L/h)	Vz/F (L)
AFQ056 15 mg	n	12	8	8
	Mean (SD)	1.40 (0.76)	75.5 (47.3)	693 (515)
	Median	1.99	56.2	489
	Min-Max	0.50-2.03	33.3-171	171-1660
	CV% mean	54.3	62.6	74.3

Cohort 2

Summary statistics for pharmacokinetic variables of secondary interest after single oral administration of 15 mg of AFQ056 Fragile X syndrome children aged between 3-4 years

Treatment		Tmax (h)	CL/F (L/h)	Vz/F (L)
AFQ056 15 mg	n	9	8	8
	Mean (SD)	0.869 (0.652)	49.9 (16.5)	362 (203)
	Median	0.550	44.2	301
	Min-Max	0.450-2.02	[24.6;75.9]	[145;749]
	CV% mean	75.0	33.0	55.9

Summary of Safety

Safety Results

Cohort 1

Incidence of adverse events by primary system organ class - n(percent) of patients (Safety analysis set)

Body system	-Period1- 15mg AFQ056 N=12 n (%)	-Period2- 20mg AFQ056 N=1 n (%)	25mg AFQ056 N=1 n (%)	50mg AFQ056 N=3 n (%)	60mg AFQ056 N=1 n (%)	100mg AFQ056 N=6 n (%)	Total N=12 n (%)
Patients with AE(s)	4(33.3)	1(100)		3(100)	1(100)	4(66.7)	9(75.0)
System organ class							
Gastrointestinal disorders	2(16.7)	1(100)		3(100)		3(50.0)	8(66.7)
Nervous system disorders		1(100)		1(33.3)		3(50.0)	5(41.7)
Psychiatric disorders	1(8.3)	1(100)			1(100)	2(33.3)	4(33.3)
Eye disorders						2(33.3)	2(16.7)
General disorders and administration site conditions						2(33.3)	2(16.7)
Infections and infestations	1(8.3)			1(33.3)			2(16.7)
Skin and subcutaneous tissue disorders						2(33.3)	2(16.7)
Renal and urinary disorders				1(33.3)			1(8.3)
Respiratory, thoracic and mediastinal disorders						1(16.7)	1(8.3)
Vascular disorders						1(16.7)	1(8.3)

Arranged in descending order of frequency (in total group) and by system organ class

Incidence of adverse events by preferred term - n(percent) of patients (Safety analysis set)

Preferred term	-Period1- 15mg AFQ056 N=12 n (%)	-Period 2- 20mg AFQ056 N=1 n (%)	25mg AFQ056 N=1 n (%)	50mg AFQ056 N=3 n (%)	60mg AFQ056 N=1 n (%)	100mg AFQ056 N=6 n (%)	Total N=12 n (%)
Patients with AE(s)	4(33.3)	1(100)		3(100)	1(100)	4(66.7)	9(75.0)
Prefered term							
Vomiting		1(100)		2(66.7)		3(50.0)	6(50.0)
Diarrhoea	1(8.3)			1(33.3)			2(16.7)
Dizziness						2(33.3)	2(16.7)
Initial insomnia		1(100)				1(16.7)	2(16.7)
Psychomotor hyperactivity		1(100)		1(33.3)			2(16.7)
Anticipatory anxiety	1(8.3)					1(16.7)	1(8.3)
Anxiety					1(100)		1(8.3)
Coordination abnormal						1(16.7)	1(8.3)
Dyskinesia				1(33.3)			1(8.3)
Eye pain						1(16.7)	1(8.3)
Feeling hot						1(16.7)	1(8.3)
Food poisoning	1(8.3)						1(8.3)
Headache						1(16.7)	1(8.3)

Hordeolum	1(8.3)					1(8.3)
Hot flush					1(16.7)	1(8.3)
Hyperhidrosis					1(16.7)	1(8.3)
Hypersomnia					1(16.7)	1(8.3)
Mydriasis					1(16.7)	1(8.3)
Nasopharyngitis				1(33.3)		1(8.3)
Non-cardiac chest pain					1(16.7)	1(8.3)
Oropharyngeal pain					1(16.7)	1(8.3)
Pollakiuria				1(33.3)		1(8.3)
Swelling face					1(16.7)	1(8.3)

Arranged in descending order of frequency by total column

Cohort 2

Incidence of adverse events by primary system organ class - n(percent) of patients (Safety analysis set)

	-Period1-	-Period2-					
Body system	15mg AFQ056 N=9 n (%)	15mg AFQ056 N=1 n (%)	20mg AFQ056 N=1 n (%)	25mg AFQ056 N=2 n (%)	50mg AFQ056 N=4 n (%)	100mg AFQ056 N=1 n (%)	Total N=9 n (%)
Patients with AE(s)	2(22.2)	1(100)	1(100)		3(75.0)	1(100)	6(66.7)
System organ class							
Psychiatric disorders			1(100)		2(50.0)		3(33.3)
Infections and infestations	2(22.2)	1(100)					2(22.2)
Nervous system disorders					1(25.0)		1(11.1)
Respiratory, thoracic and mediastinal disorders						1(100)	1(11.1)
Skin and subcutaneous tissue disorders			1(100)				1(11.1)

Arranged in descending order of frequency (in total group) and by system organ class

Incidence of adverse events by preferred term - n(percent) of patients (Safety analysis set)

	-Period1-	-Period2-					
Preferred term	15mg AFQ056 N=9 n (%)	15mg AFQ056 N=1 n (%)	20mg AFQ056 N=1 n (%)	25mg AFQ056 N=2 n (%)	50mg AFQ056 N=4 n (%)	100mg AFQ056 N=1 n (%)	Total N=9 n (%)
Patients with AE(s)	2(22.2)	1(100)	1(100)		3(75.0)	1(100)	6(66.7)
Preferred term	-	-	-				
Insomnia	-	-	-		2(50.0)		2(22.2)
Gastrointestinal viral infection	-	1(100)	-				1(11.1)
Infected bites	1(11.1)	-	-				1(11.1)
Initial insomnia	-	-	1(100)				1(11.1)
Nasal congestion	-	-	-			1(100)	1(11.1)
Pharyngitis	1(11.1)	-	-				1(11.1)
Rash	-	-	1(100)				1(11.1)
Somnolence	-	-	-		1(25.0)		1(11.1)
Urticaria	-	-	1(100)				1(11.1)

Serious Adverse Events and Deaths:

There were no serious adverse events or deaths reported in this study.

Other Relevant Findings

Conclusion:

Pharmacokinetics

Period 1:

- The geometric mean for Cmax and AUC0-12h was 43.1 ng/mL and 168 ng.h/mL, respectively, in cohort 1. The corresponding PK parameters were 70 ng/mL and 246 ng.h/mL, respectively, in cohort 2.

Period 2:

- Dose normalized geometric mean of Cmax,ss and AUC0-12h,ss of AFQ056 was 3.65 ng/mL/mg and 13.1 ng.h/mL/mg, respectively for cohort 1 and 3.77 ng/mL/mg and 19.5 ng.h/mL/mg of Cmax,ss and AUC0-12h,ss of AFQ056, respectively for cohort 2.
- Geometric means of dose normalized Cmax,ss and AUC0-12h,ss of AFQ056 in period 2 were in similar range between cohort 1 and cohort 2.

General aspects:

- The differences of PK parameters between cohort 1 and 2 appear to be within the inter-individual variability of AFQ056 PK determined in previous clinical studies.

  The dose normalized Cmax and AUC data obtained at a single dose of 15 mg in this population of 3-11 year-old children with Fragile X syndrome were similar compared to historical data from adolescents with Fragile X syndrome from the Novartis trial CAFQ056B2131.

- PK profile of oral suspension formulation used in this study has been previously shown to be comparable to the PK profile of MF capsule (AFQ056A2166) which is used for the adult and adolescent population in pivotal studies.

Safety and tolerability

- In this study population of patients with Fragile X syndrome aged 3-11 years, AFQ056 was safe and provided an acceptable tolerability at a single dose of 15mg and at individualized multiple doses of 1.25 mg/kg, 50 mg or 100 mg b.i.d. over seven days (cohort 1) or eight days (cohort 2), respectively.
- The concept of metabolic phenotyping (i.e. dose individualization) is believed to have contributed to the good safety and tolerability of AFQ056.

Date of Clinical Trial Report

22-Jan-2014

Date Inclusion on Novartis Clinical Trial Results Database

22 May 2014

Date of Latest Update

NOVARTIS

Clinical Trial Results Database