KalVista Pharmaceuticals

KVD001 Phase 2 Clinical Trial

December 9, 2019

KVD001 Phase 2 Clinical Trial Design

Subjects

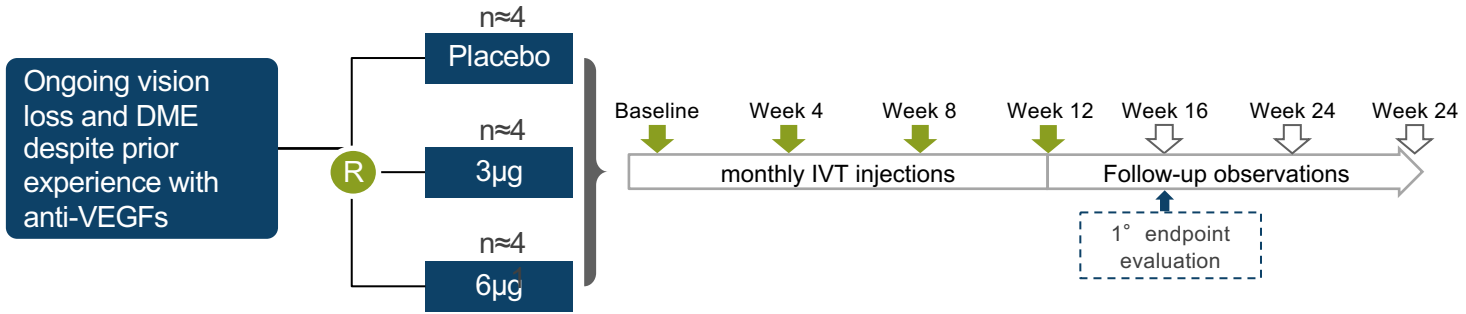
- Adult male or female subjects with confirmed DM (type I or II)
- Presence of ciDME (F: > 305µm; M: > 320 µm)
- Ongoing vision loss (20/40 or worse) despite prior anti-VEGF treatment (within 36 months of Day 1)

Setup

- 38 US sites
- Double-masked, parallel group, 1:1:1 randomization, stratified for BCVA, CST at baseline
- 4 monthly injections with safety follow-up

Stats

- N=41 per arm, power 80%, with change in 5±7.5 letters between active and sham, α=0.05
- Primary analysis: ANCOVA, LSmeans change in BCVA at week 16 from baseline, active vs. sham, adj. for BCVA at baseline, last-observation carried forward



KVD001 Phase 2 Clinical Trial Endpoints

1° efficacy endpoint

- Change from baseline BCVA letter count via ETDRS at Week 16

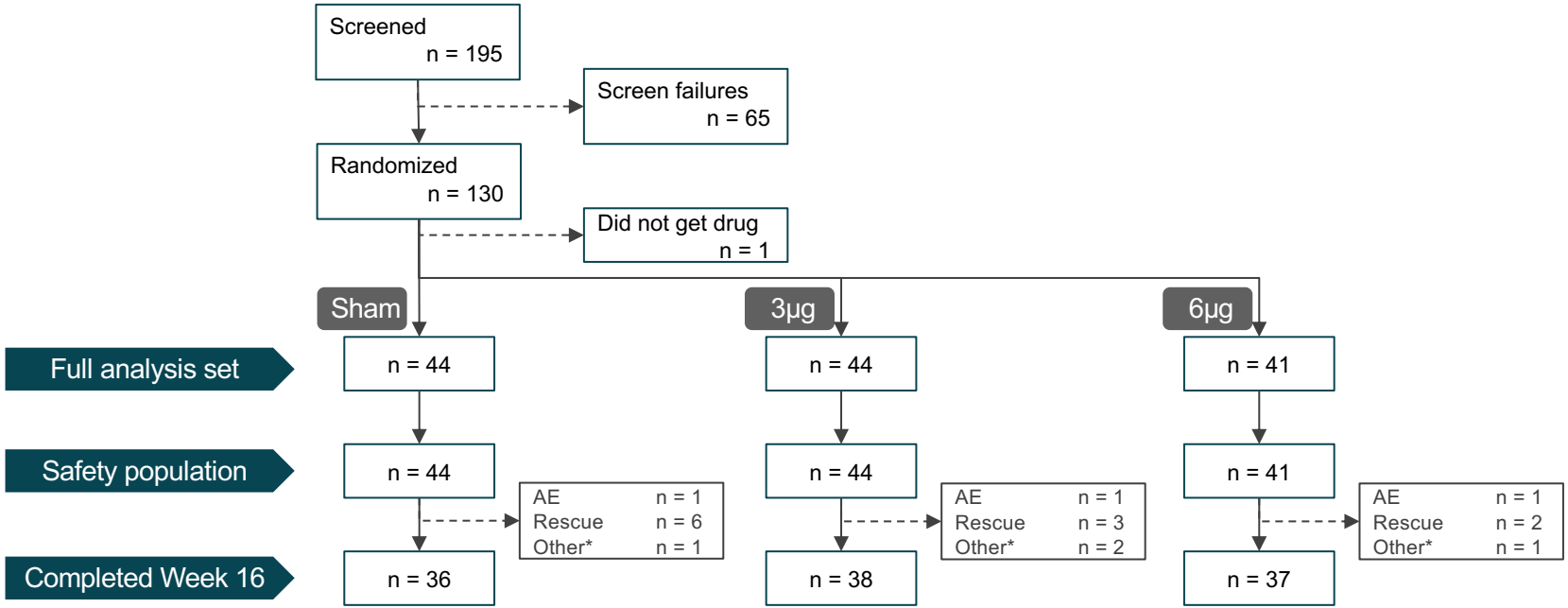
2° efficacy endpoints

- Change from baseline CST via SD-OCT
- Proportion of eyes w/≥ 2 step DRSS improvement from baseline
- Change from baseline BCVA via ETDRS at wk 4, 8, 12, 20 and 24
- Proportion of eyes with ≥5, ≥10 and ≥15 BCVA letter change from baseline (gain and loss)

Safety endpoints

- Adverse events
- Ophthalmic exam and physical exam findings
- Laboratory test results
- Vital signs

CONSORT Diagram & Analysis Populations



*Other includes reason for discontinuation from study reported as: subject compliance, withdrawal by subject or other

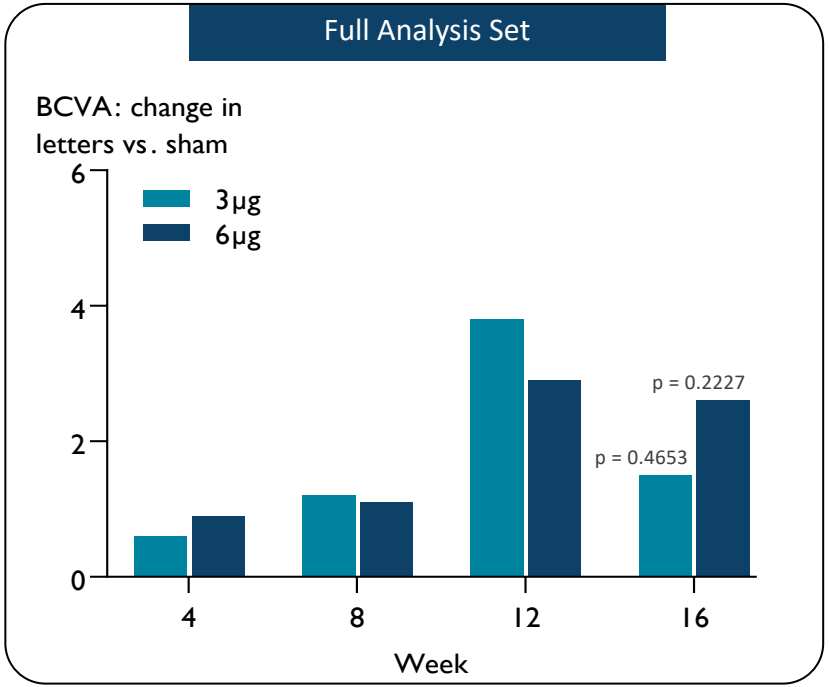
Baseline Population Characteristics

Variable		Sham n = 44		KVD001 3µg n= 44		KVD001 6µg n= 41		Total n = 129	
Age	mean (SD)	64.6	(8.6)	61.1	(10.7)	63.2	(9.6)	63.0	(9.7)
Sex	female / male n (%)	23/21	(52.3/47.7)	20/24	(45.5/54.5)	15/26	(36.6/63.4)	58/71	(45.0/55.0)
Race	white n (%)	37	(84.1)	37	(84.1)	37	(90.2)	111	(86.0)
	black n (%)	3	(6.8)	5	(11.4)	4	(9.8)	12	(9.3)
	other n (%)	4	(9.1)	2	(4.6)	0	(0)	6	(4.7)
BMI	mean (SD)	31.6	(7.0)	33.0	(7.7)	31.7	(5.4)	32.1	(6.8)
HbA1c	mean (SD)	7.5	(1.2)	7.6	(1.3)	8.0	(1.5)	_	_
Duration of DME (yrs)	mean (SD)	1.4	(1.3)	1.0	(0.9)	1.0	(1.2)	1.1	(1.1)
Type 2 DM	n (%)	44	(100)	40	(90.9)	40	(97.6)	124	(96.1)

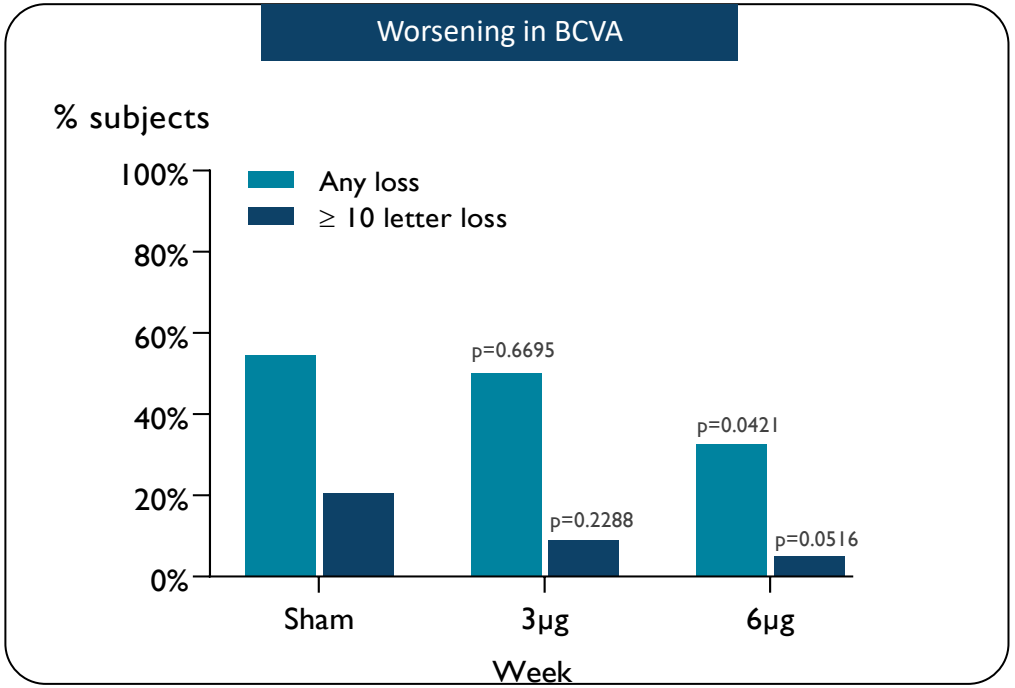
Baseline Ocular Characteristics

Variable		Sham n = 44		KVD001 3µg n= 44		KVD001 6µg n= 41		Total n = 129	
BCVA (letters)	mean (SD)	60.7	(7.4)	58.8	(12.5)	58.0	(12.8)	59.2	(11.1)
BCVA > 55	n (%)	33	(75.0)	31	(70.5)	30	(73.2)	94	(72.9)
CST (µm)	mean (SD)	500	(131)	540	(166)	512	(134)	517	(145)
CST ≤ 450 µm	n (%)	17	(38.6)	17	(38.6)	15	(36.6)	49	(38.0)
Prior IVT anti-VEGF	mean (SD)	8.0	(4.0)	7.4	(4.1)	5.9	(3.0)	7.1	(3.8)
Prior IVT steroid	n (%)	13	(29.5)	12	(27.3)	10	(24.4)	35	(27.1)

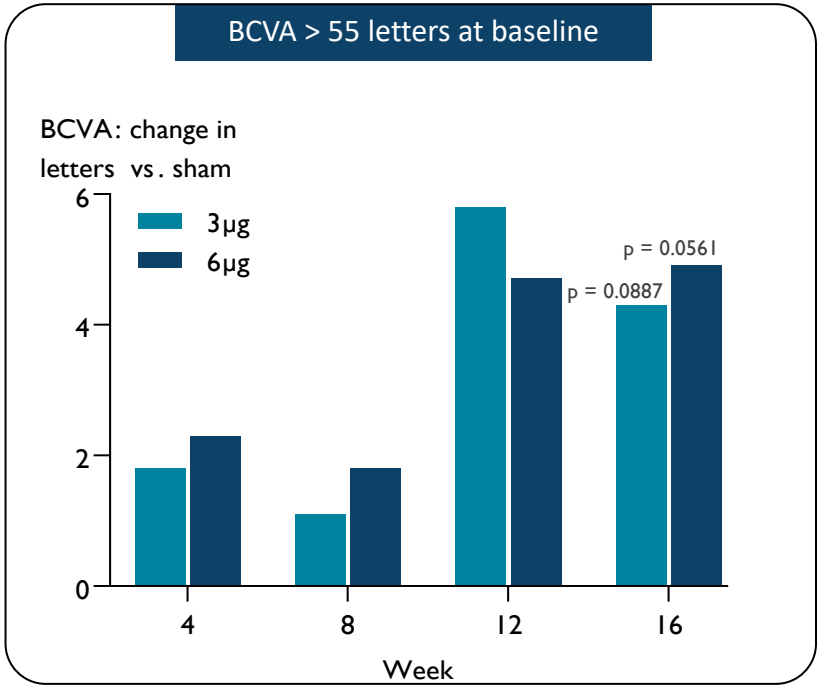
Study Did Not Meet the Primary BCVA Endpoint



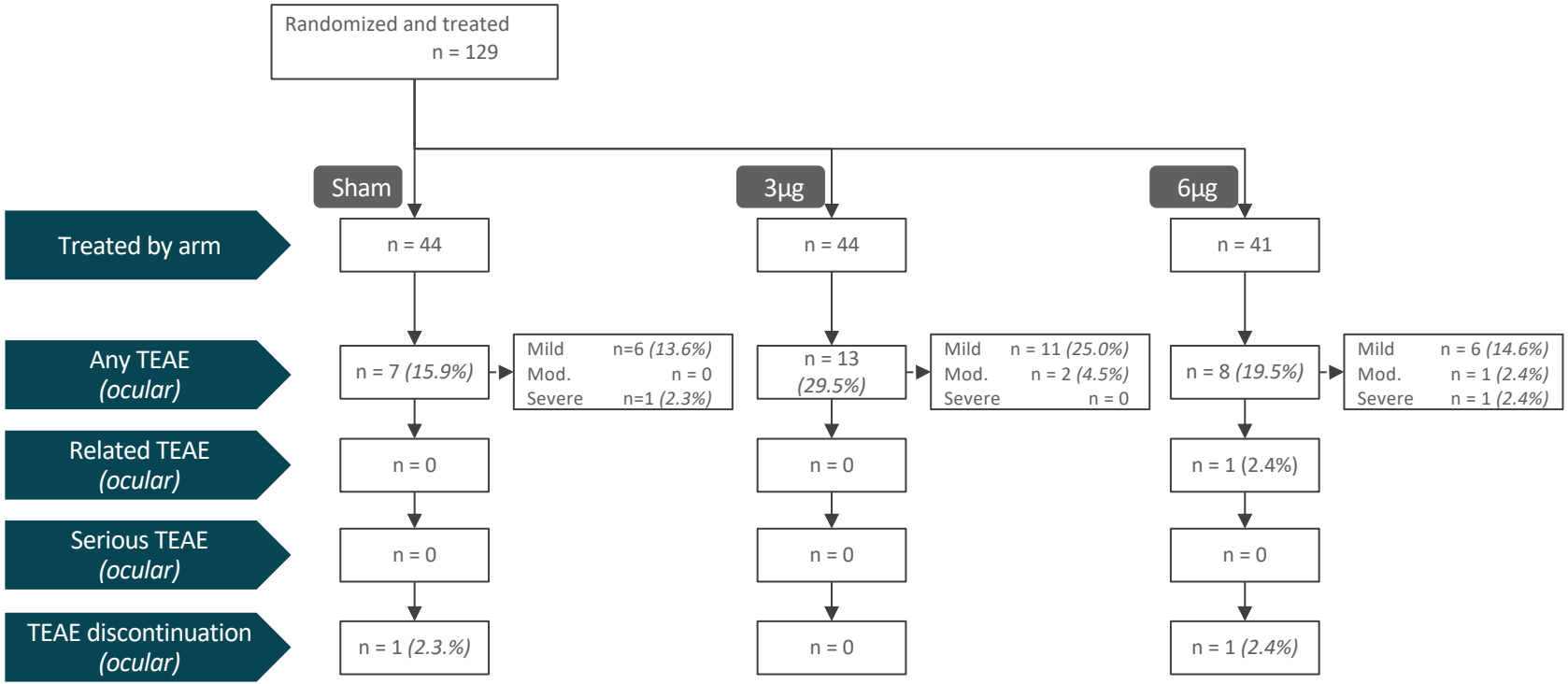
Vision Loss Protected In a Dose Responsive Manner



More Robust Response in Subjects with Baseline Vision of >55 Letters



KVD001: Generally Safe and Well Tolerated



KVD001 Phase 2 Clinical Trial Conclusions

- The study did not meet the primary or secondary efficacy endpoints of changes in BCVA, CST, or DRSS
- The trial population had shown poor BCVA response to prior anti-VEGF
- KVD001 showed dose responsive protection from vision loss
- Patients with less severe vision loss experienced more robust treatment benefit
  - This represents >70% of the total trial population
- KVD001 was generally safe and well tolerated
- The results support further study of KVD001 as a treatment for DME
  - Higher doses and combination with anti-VEGF already enabled
  - Potential for orally delivered molecules to deliver differentiated treatment option

KalVista Pharmaceuticals

NASDAQ: KALV