



Corporate Presentation

November 2021

Forward-Looking Statements

This presentation contains statements about our future expectations, plans and prospects that constitute forward-looking statements for purposes of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including risks relating to: both our and our collaborators' ability to successfully research, obtain regulatory approvals for, develop and commercialize products based upon our technologies; our ability to obtain and maintain proprietary protection for our technologies and product candidates; our reliance on third parties to manufacture our preclinical and clinical drug supplies; competitive pressures; our ability to obtain and maintain strategic collaborations; compliance with our in-license agreements; our ability to successfully execute on, and receive favorable results from, our proprietary drug development efforts; market acceptance of our drug candidates; retaining members of our senior management; and our ability to raise additional funds to finance our operations.

The forward-looking statements included in this presentation represent our views as of the date of this presentation. We anticipate that subsequent events and developments will cause our views to change. While we may elect to update these forward-looking statements in the future, we specifically disclaim any obligation to do so. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this presentation.

For more information regarding risks and uncertainties that could affect the results of our operations or financial condition review our filings with the Securities and Exchange Commission (in particular, our most recent Annual Report on Form 10-K and any subsequently filed Quarterly Reports on Form 10-Q).

Investment Highlights

- **Focused on novel therapeutics for metabolic and endocrine diseases**
 - Clinical programs demonstrate best-in-class efficacy data
- **Metabolic Disease Program: VK2809 for NASH**
 - Novel, selective thyroid receptor- β (TR β) agonist
 - Phase 2a results demonstrate significant reduction in liver fat content and lipids
 - Phase 2b VOYAGE trial in biopsy-confirmed NASH ongoing
- **Rare Disease Program: VK0214 for X-ALD**
 - Novel, selective thyroid receptor- β (TR β) agonist
 - In vivo data show improvement in key biomarkers
 - Phase 1b study in patients underway
- **Other Pipeline Programs: Musculoskeletal and metabolic disorders**

Pipeline Overview

Development Programs	Indication	Stage of Development				Status
		Preclin	Phase 1	Phase 2	Phase 3	
VK2809 (TR β agonist)	<i>NASH</i>	[Progress bar: Preclin, Phase 1, Phase 2]				Phase 2b VOYAGE trial ongoing
VK0214 (TR β agonist)	<i>X-ALD</i>	[Progress bar: Preclin, Phase 1]				Phase 1b ongoing
Other Programs						
VK5211 (SARM)	<i>Hip fracture, muscle wasting</i>	[Progress bar: Preclin, Phase 1, Phase 2]				Phase 2 completed
VK0612 (FBPase inhibitor)	<i>Type 2 Diabetes</i>	[Progress bar: Preclin, Phase 1, Phase 2]				Phase 2a completed
VK1430 (DGAT-1 inhibitor)	<i>Hypertriglyceridemia, NASH</i>	[Progress bar: Preclin]				Preclinical



Metabolic Disease Program
VK2809: Selective Thyroid Receptor- β Agonist
Liver Disorders

Metabolic Disease Program: Selective Thyroid- β Agonists

- **Proprietary platform for small molecule thyroid hormone mimetics**
 - Highly tissue and receptor selective
 - Produce potent lipid reductions in animals and humans
 - Unique chemical scaffolds, expected wider safety window vs. other approaches
- **Biological profiles suggest potential benefit in multiple indications**
 - Broad: NASH, hypercholesterolemia, dyslipidemia
 - Rare: X-linked adrenoleukodystrophy (X-ALD), other
- **Lead molecules VK2809, VK0214**
 - Oral, once-daily formulations
 - VK2809: Phase 2b ongoing, biopsy-confirmed NASH
 - VK0214: Phase 1b ongoing, AMN

Thyroid Hormone Receptor Overview

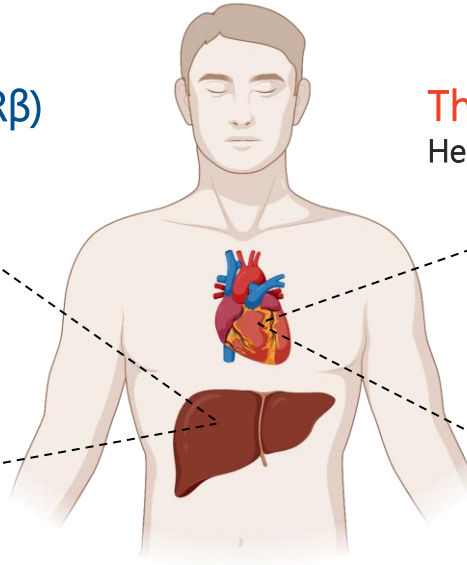
Nuclear hormone receptors: 2 main types

Thyroid hormone receptor beta (TR β)

Liver

Positive effects

- Regulates lipid metabolism
- Reduces LDL-C, triglycerides, atherogenic proteins
- Improves metabolic control



Thyroid hormone receptor alpha (TR α)

Heart, skeletal muscle

Negative effects

- Proarrhythmic potential
- Elevates heart rate
- Bone/cartilage effects

Therapeutic goal, lipid setting: Beta receptor selectivity, minimize alpha effects

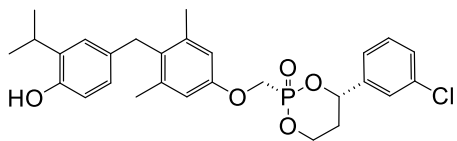
Thyroid Receptor β Agonists for NAFLD and NASH



- β -Receptor: Key role in lipid metabolism; systemic and liver-specific effects
- Receptor localized to liver, limited ex-hepatic expression
- In vivo evidence suggests β -activation provides anti-fibrotic benefits
- Clinical data indicate correlation between reduced liver fat, improvement in NAS

An agent that reduces liver fat, improves systemic lipids, and antagonizes fibrotic signaling could provide multi-pronged benefits in NASH

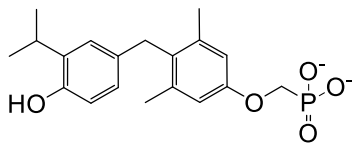
VK2809: Unique Liver-Targeted Characteristics



VK2809, Novel Prodrug

Following oral dosing:

- Cyp3A4-mediated cleavage of prodrug
- 3A4 is primarily expressed in liver
- Results in targeted delivery of drug to liver



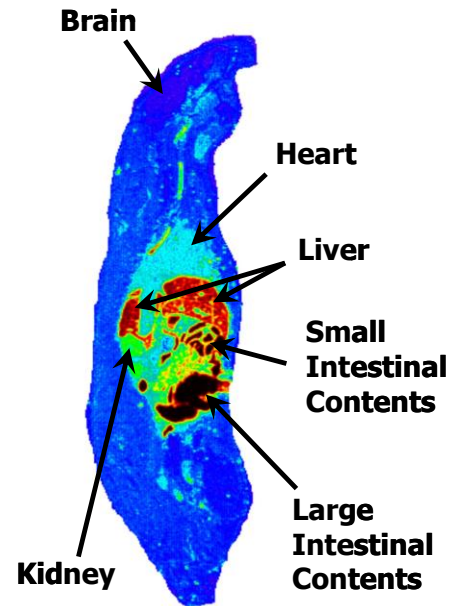
VK2809A, Potent TR β Agonist, 2.2 nM Ki

^{14}C QWBA (4 h)

High



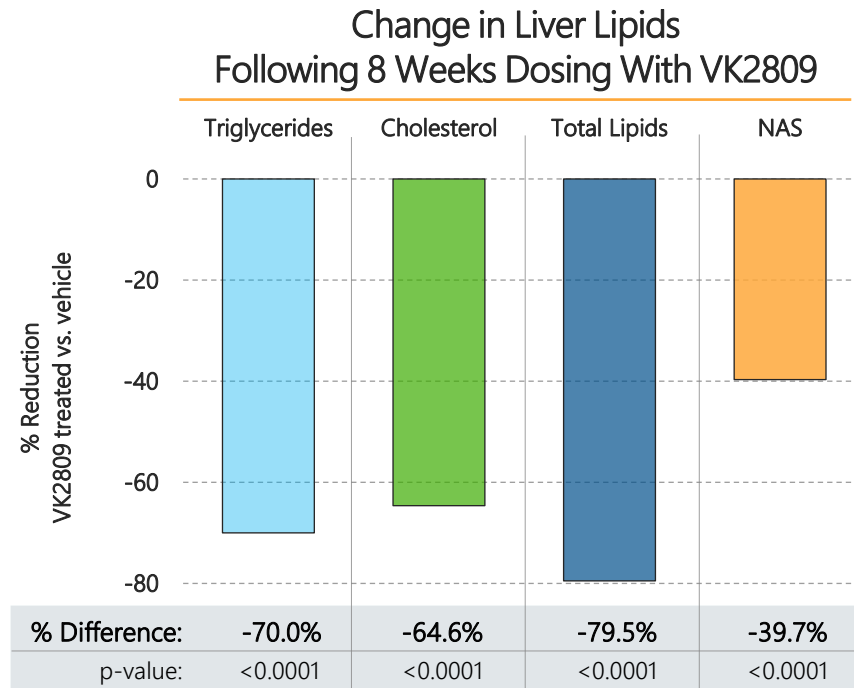
Low



Selective activation, differentiated chemistry lends VK2809 liver selectivity; potentially minimizes risk of systemic effects

VK2809 Significantly Reduces Steatosis in Diet-Induced NASH

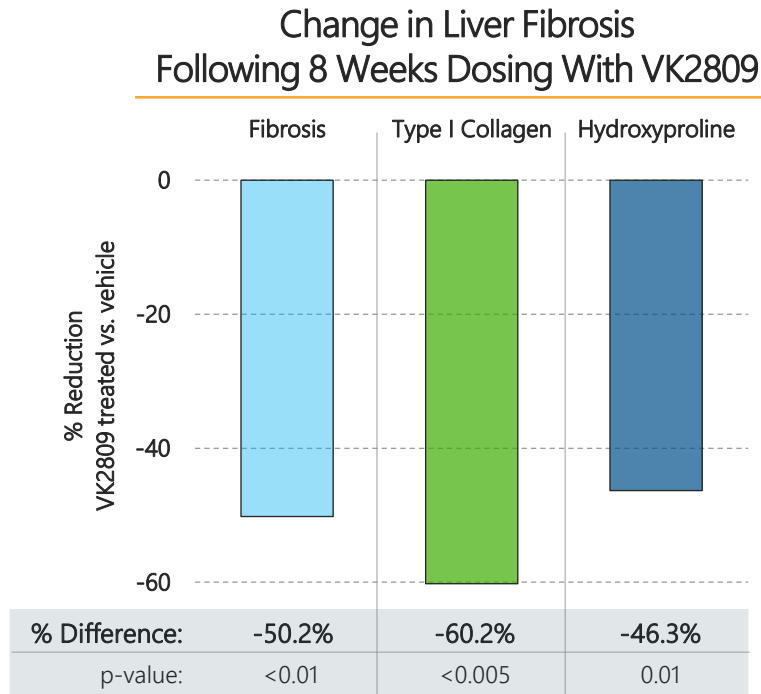
- Evaluation in biopsy-confirmed diet-induced NASH model
 - Rodent model designed to reflect progression of disease in humans
 - Animals biopsied pre-study; only those with NASH *and* fibrosis selected
 - VK2809 dosed once-daily for 8 weeks



Treatment with VK2809 significantly improves lipids, steatosis, NAS at 8 weeks; well-tolerated with no evidence of toxicity

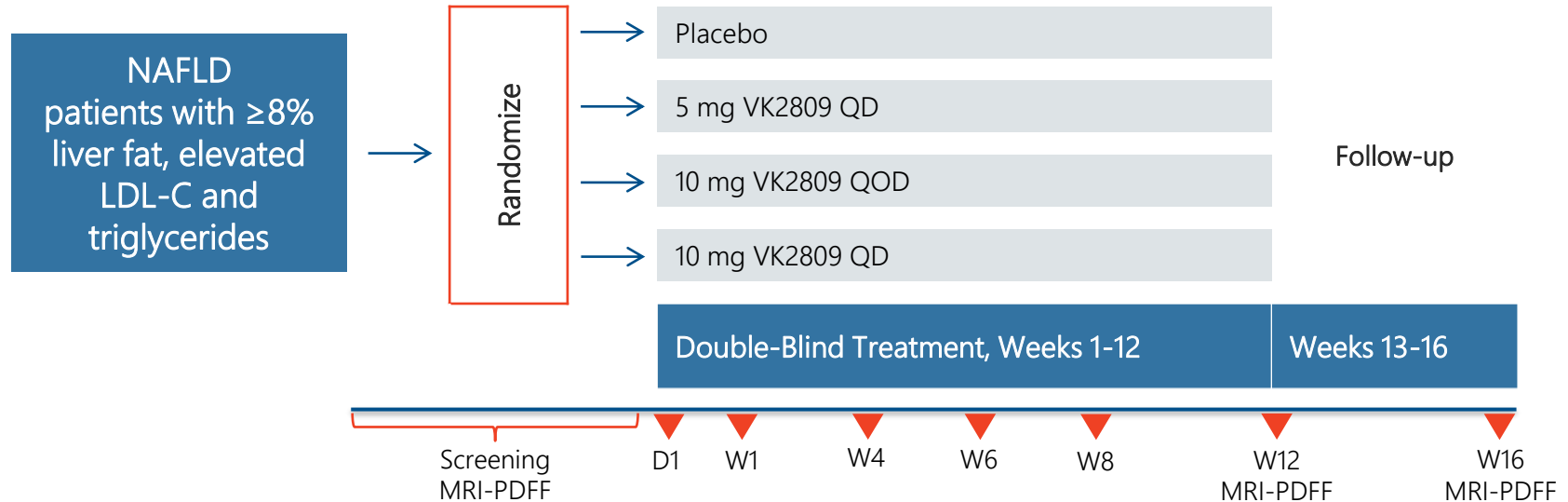
VK2809 Improves Fibrosis in Diet-Induced NASH Model

- Significant reductions in fibrosis, collagen, hydroxyproline after 8 weeks
- Supports thesis that selective TR β activation produces broad metabolic benefits



VK2809 significantly improved NASH and fibrosis in this model

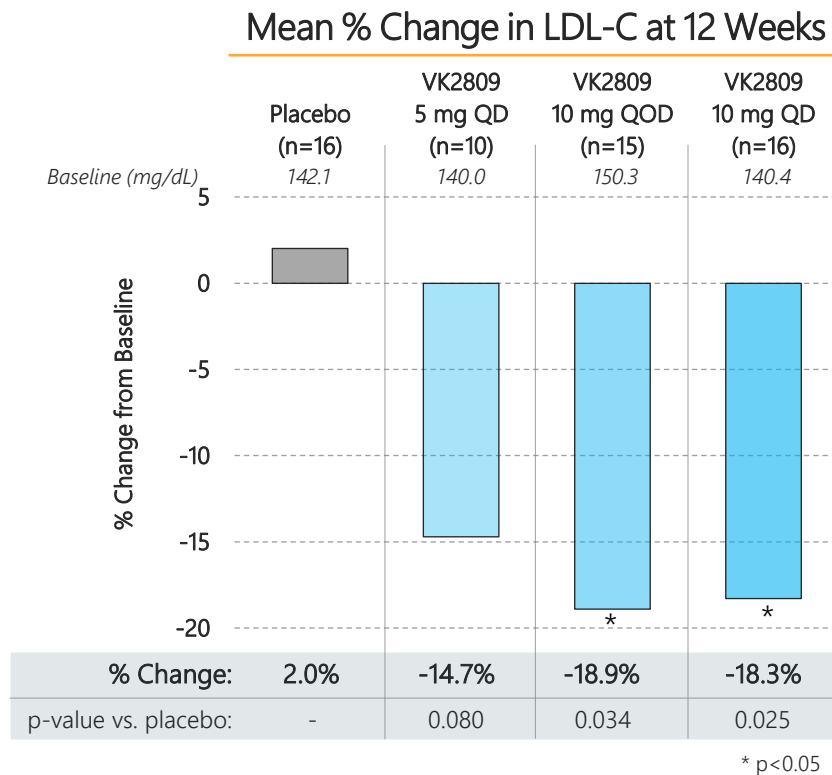
VK2809-201: Phase 2a Study Design



- Multi-arm, dose-ranging, 12 week Phase 2a trial
 - **Primary endpoint:** Change in LDL-C vs. placebo
 - **Secondary endpoint:** Change in liver fat by MRI-PDFF
 - **Exploratory endpoints:** Changes in atherogenic proteins

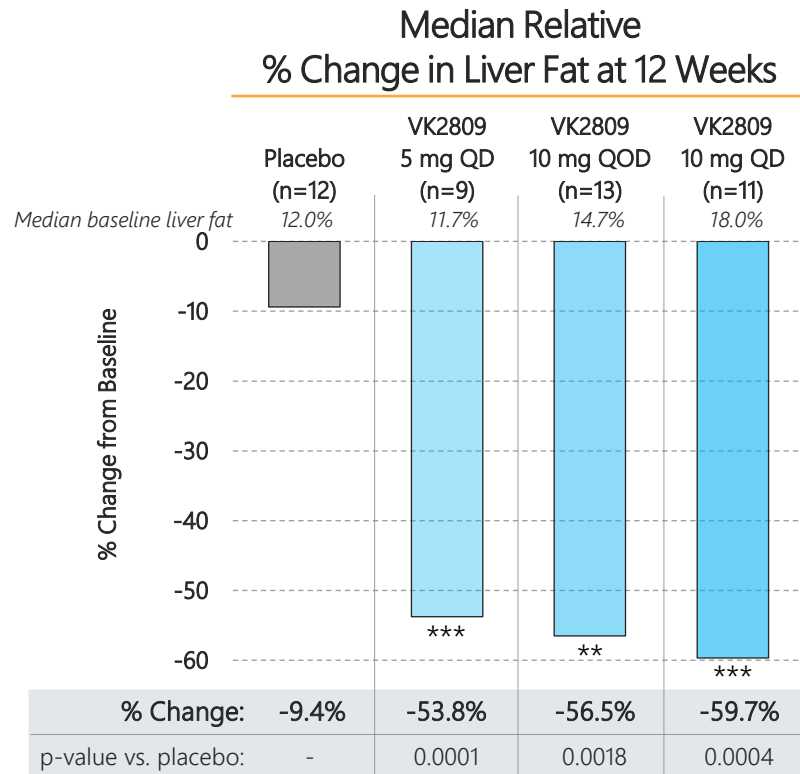
VK2809 Significantly Reduced LDL-C After 12 Weeks

- All VK2809 cohorts statistically significantly reduced vs. baseline
- Placebo-adjusted change from baseline
 - 5 mg QD: -23.7 mg/dL
 - 10 mg QOD: -27.1 mg/dL
 - 10 mg QD: -28.3 mg/dL



VK2809 Produced Significant Relative Reductions in Liver Fat

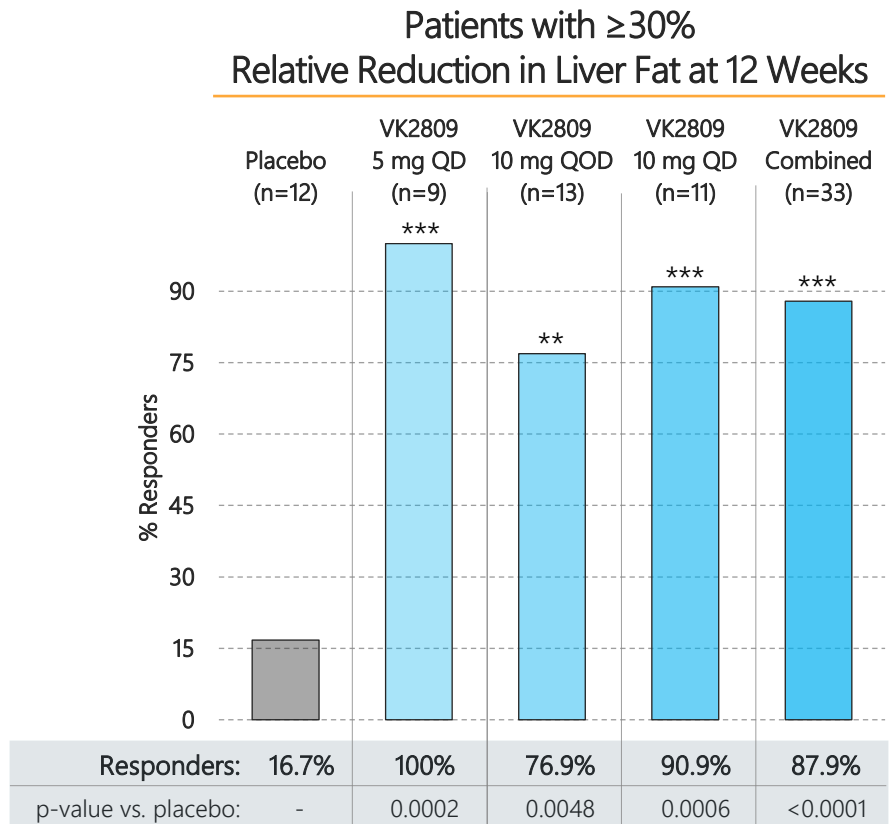
- Significant Relative Reductions from Baseline in Liver Fat by MRI-PDFF
- Maximal reductions at Week 12
 - 5 mg QD: 78%
 - 10 mg QOD: 72%
 - 10 mg QD: 76%



*p<0.05; **p<0.01; ***p<0.001

VK2809 Cohorts Demonstrated High Relative Response Rates

- Up to 100% of VK2809 patients experienced response, as defined by $\geq 30\%$ decrease in liver fat at Week 12
- Combined VK2809 cohorts demonstrated 88% response rate
- 70% of all patients receiving VK2809 demonstrated liver fat reductions $\geq 50\%$
- Reduction in liver fat correlated with improved odds of long-term histology benefit¹

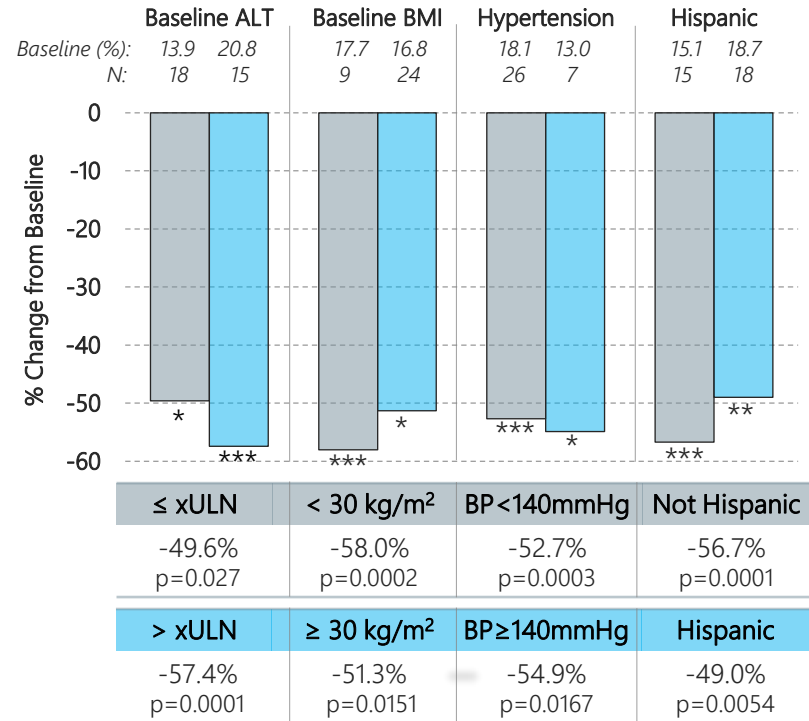


p<0.01; *p<0.001

Consistent Week 12 MRI-PDFF Reductions Across Key Subsets

- Consistent MRI-PDFF reduction independent of risk factors:
 - ALT >xULN
 - BMI ≥ 30 kg/m²
 - Baseline BP ≥ 140 mmHg
 - Hispanic ethnicity

Mean % Change in Liver Fat at 12 Weeks in VK2809 Treated Patients



*p<0.05; **p<0.01; ***p<0.001

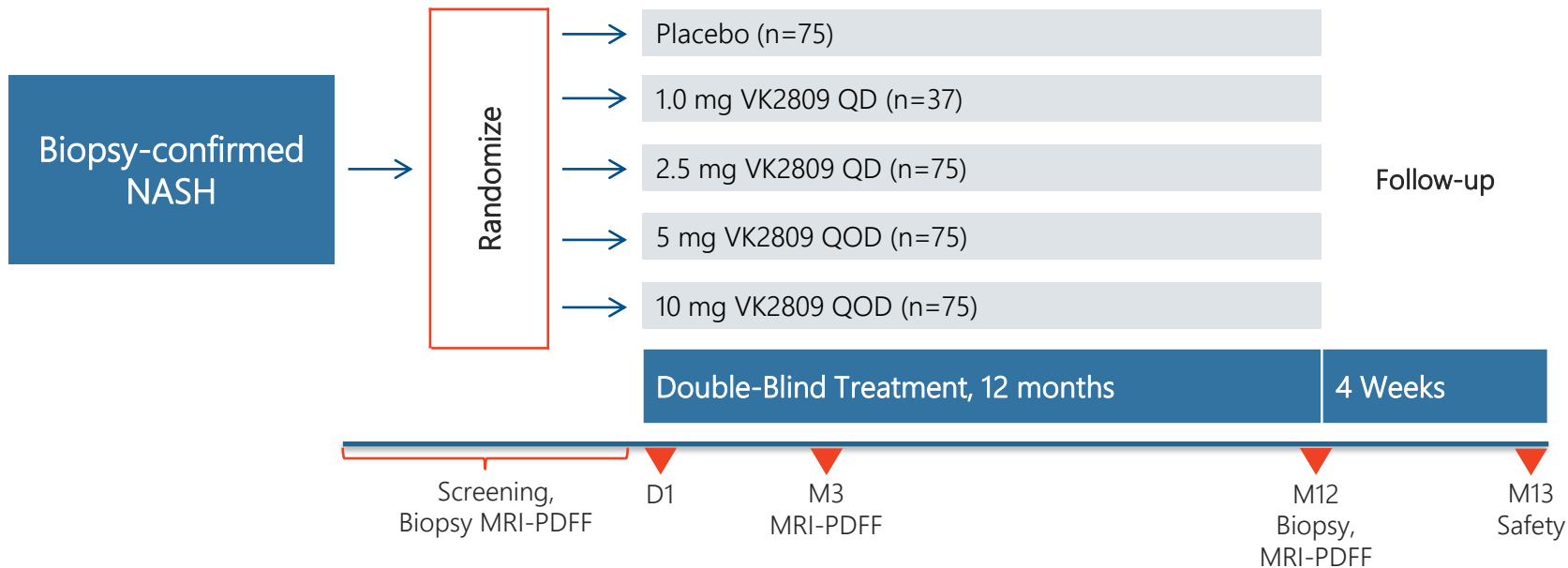
VK2809: Phase 2a Summary and Conclusions

- VK2809 produced robust reduction in liver fat on MRI-PDFF in NAFLD patients after 12 weeks of dosing
 - Consistent effects observed across common NASH risk factors
 - Liver fat reduction maintained 4 weeks post-dosing
 - VK2809 produced significant reduction in plasma lipids, suggesting long-term cardiovascular benefit
 - VK2809 was safe and well-tolerated in this 12-week study
- 88% of patients receiving VK2809 experienced $\geq 30\%$ reduction in liver fat content, including all patients receiving 5 mg doses; 70% experienced reductions $\geq 50\%$
- Elevated ALT, BMI, hypertension, Hispanic ethnicity
- 70% of VK2809 patients remained responders 28 days after conclusion of dosing
- LDL-C, triglycerides, and atherogenic proteins Apo B, Lp(a)
- No SAEs observed, discontinuations well-balanced across cohorts



VK2809: Phase 2b VOYAGE Study

VOYAGE Study: 12-Month Phase 2b Study of VK2809



- Multi-arm, dose-ranging, 12-month Phase 2 trial
 - **Primary endpoint:** Change in MRI-PDFF vs. placebo at 3 months
 - **Secondary endpoint:** Change in histology at 12 months (NAS, fibrosis markers, etc.)

VOYAGE Study: 12-Month Phase 2b Study of VK2809

- Key inclusion criteria:
 - Biopsy-confirmed NASH with NAS ≥ 4
 - Liver fat content $\geq 8\%$
 - F2-F3 fibrosis, up to 25% F1
- Primary endpoint: Change in liver fat content at week 12
- Secondary, exploratory endpoint: Change in histology at 12 months

VK2809 Competitive Advantages

- Currently >40 NASH programs in Phase 2 or Phase 3 development
- What differentiates VK2809 from the crowd?

- Orally available

Preferred route of administration for chronic therapy

- Liver-targeted

Reduces risk of undesired effects in other tissues

- Potently reduces liver fat

Weight loss and reduced liver fat correlate with NASH resolution, improved fibrosis markers

- Reduces systemic lipids, may improve overall metabolic profile

Bodes well for potential long-term CV benefit
No elevations in other lipids that may require polypharmacy

- Well tolerated

No GI impact, no pruritis or other tolerability issues to date



Rare Disease Program

VK0214

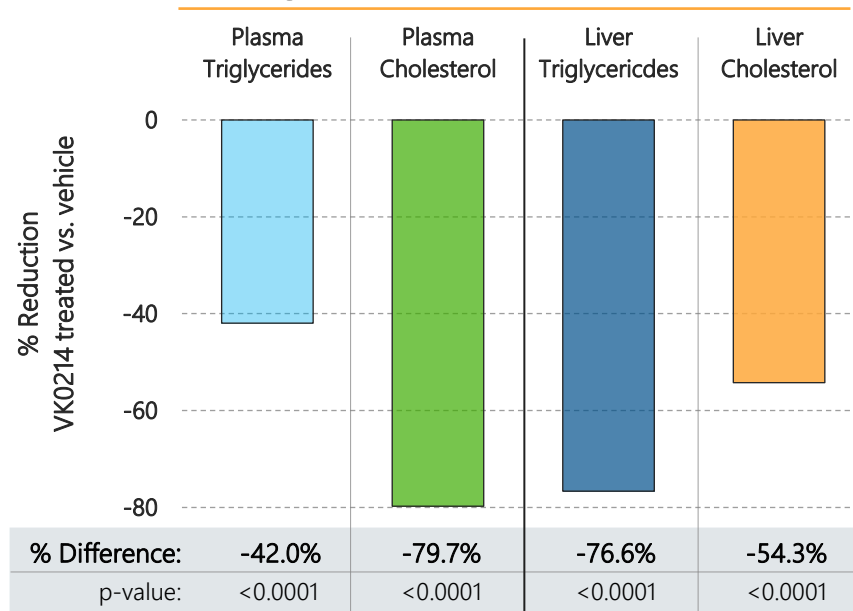
X-Linked Adrenoleukodystrophy

VK0214: Summary Profile

VK0214

- Potent small molecule thyroid receptor beta agonist
- 8 nM Ki at TR β receptor
- >20:1 selective for β : α
- Oral formulation, once-daily dosing
- Robust lipid lowering effects in multiple models

Change in Lipids Following 12 Weeks of Dosing With VK0214; Rodent NASH model



Demonstrates in vivo lipid reducing efficacy comparable to VK2809

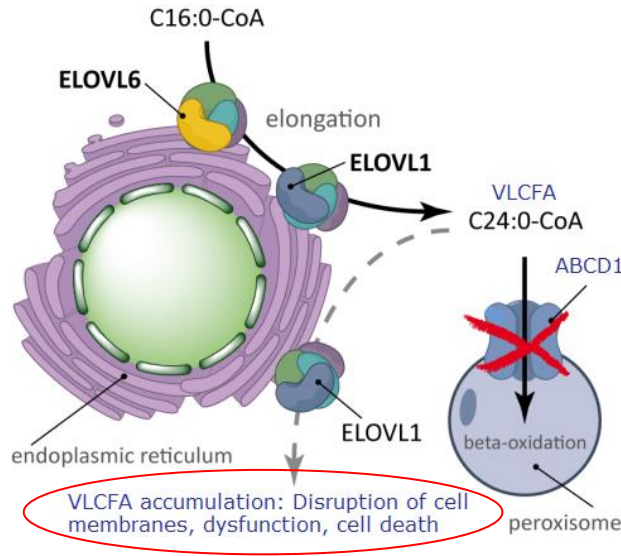
VK0214 for X-ALD

- **X-Linked adrenoleukodystrophy (X-ALD)**
 - Orphan neurodegenerative disorder
 - X-linked: Carried by females, primarily manifesting in males
 - No cure, no approved therapy
- **Most severe form: Cerebral ALD**
 - Rapidly progressive inflammatory demyelination; disruption of BBB
 - Affects ~35% before age 12 (CCALD), ~20% between age 20 – 35 (CALD)
 - Deterioration in speech, cognition; vegetative state within 3-5 years
- **Most common form: Adrenomyeloneuropathy (AMN)**
 - Affects spinal cord, motor neurons; no inflammatory component or brain involvement
 - Affects nearly all adult patients; considered “default” manifestation of ALD
 - Progressive motor impairment; wheelchair confinement, leg paralysis common

TR β : X-Linked Adrenoleukodystrophy

Caused by mutation in gene for the ATP-Binding Cassette transporter D1 (ABCD1)

- Peroxisomal transporter of very long chain fatty acids (VLCFA)



ABCD1: Normal function to transport VLCFA into peroxisome for degradation

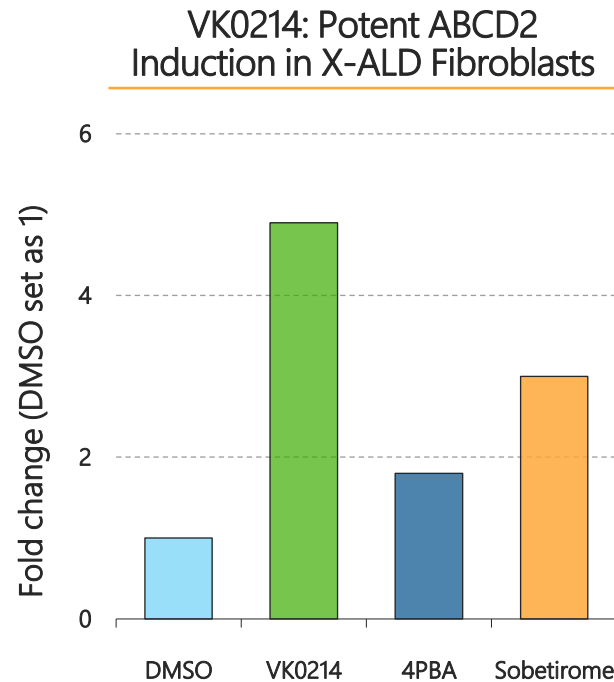
X-ALD: Defective ABCD1 leads to accumulation of VLCFA in tissues

High VLCFA levels disrupt cell membranes; inflammatory demyelination in brain tissue; motor neuron deterioration

TR β Agonists: Stimulate expression of compensatory transporters ABCD2, 3; may mitigate VLCFA elevation

Strong Rationale for TR β Role in X-ALD

- Alternative VLCFA transporters ABCD2,-3 are induced by TR β receptor
 - Mechanistically compensate for ABCD1 deficiency
- Over-expression of ABCD2 corrects VLCFA elevation *in vitro* and *in vivo*
- TR β agonists such as VK0214 hold promise
- *In vitro* PoC established in X-ALD fibroblasts

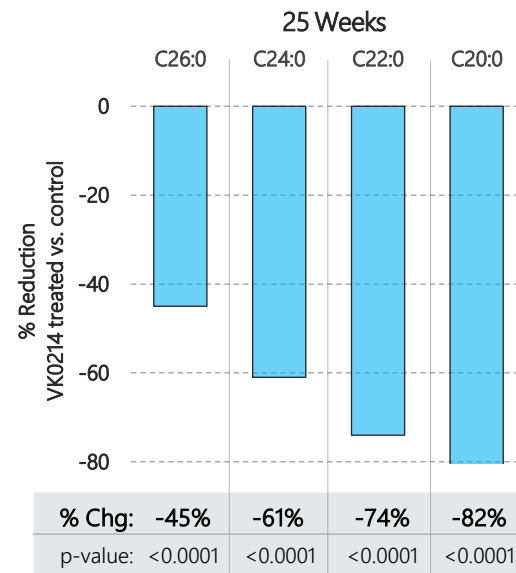
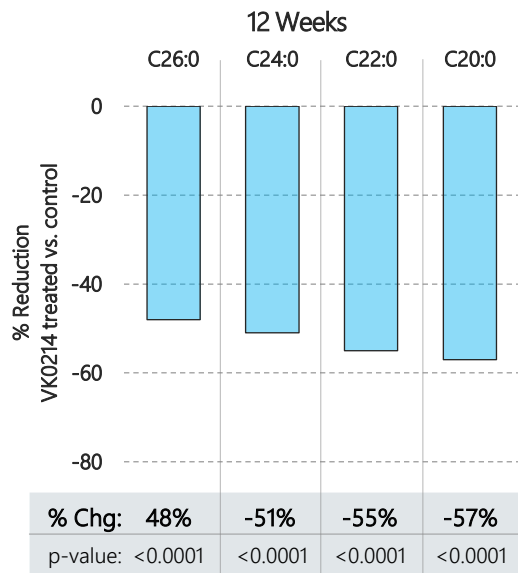
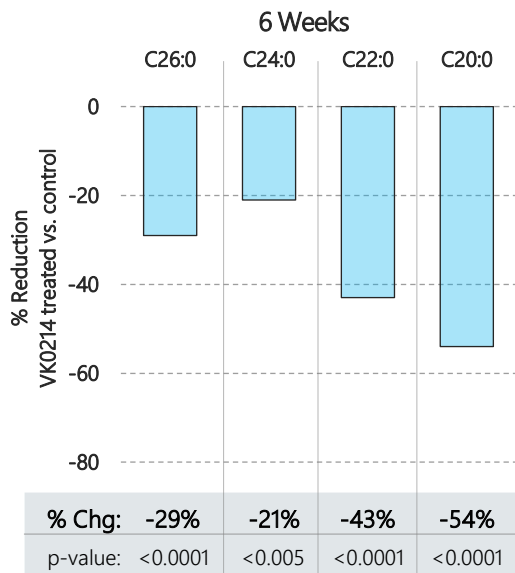


VK0214 successfully induces ABCD2 expression in human X-ALD cells

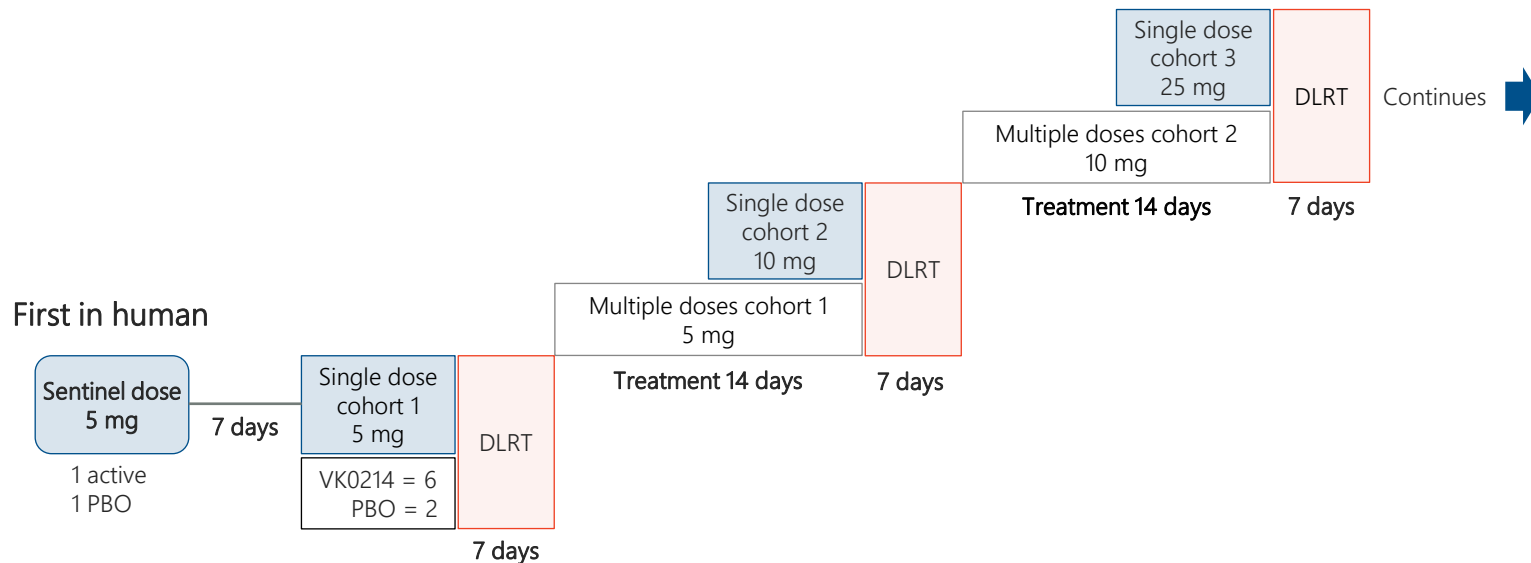
VK0214: *In Vivo* Proof-of-Concept Data, ABCD1 KO Mouse

- ABCD1 Knockout model: Mimics biochemical features of human X-ALD
- VK0214: Durable and progressive reductions in plasma VLCFAs
 - Tissue effects suggest encouraging CNS activity following long-term exposure

Reductions in Plasma VLCFA-LPC, ABCD1 Knockout Model



VK0214: Phase 1 SAD-MAD Study Design

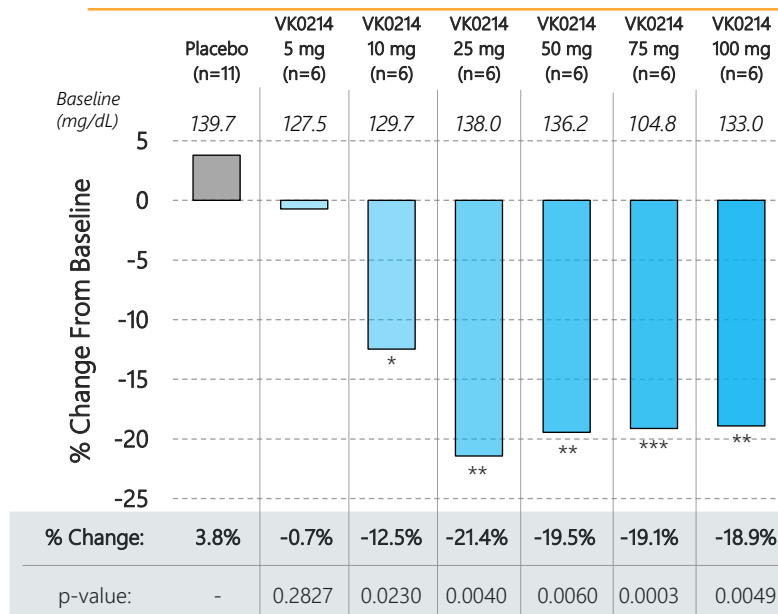


- Concurrent SAD/MAD follow “stacked” cohort approach
 - MAD commences once prior SAD cohort demonstrates safety
- Primary objective: Safety, tolerability, pharmacokinetics

VK0214 LDL-C Effect at 14 Days

- Reduction in LDL-C similar to observations with VK2809
- Initial effect observed @ ~10 mg
- Appears to plateau at approximately 20% reduction from baseline

Mean % Change in LDL-C at Day 14



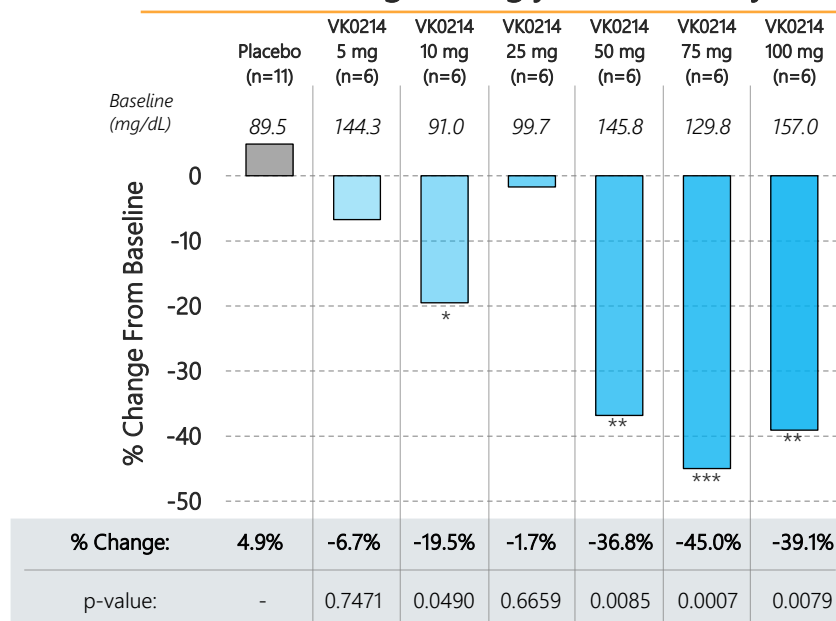
*p<0.05; **p<0.01; ***p<0.001

~20% reduction from baseline consistent with TR β agonist mechanism

VK0214 Triglyceride Effect at 14 Days

- Triglyceride reduction mirrors reported efficacy from TR β agonism
- Onset likely between 10 mg – 25 mg
- Plateau effect at approximately 35% to 40% reduction from baseline

Mean % Change in Triglycerides at Day 14



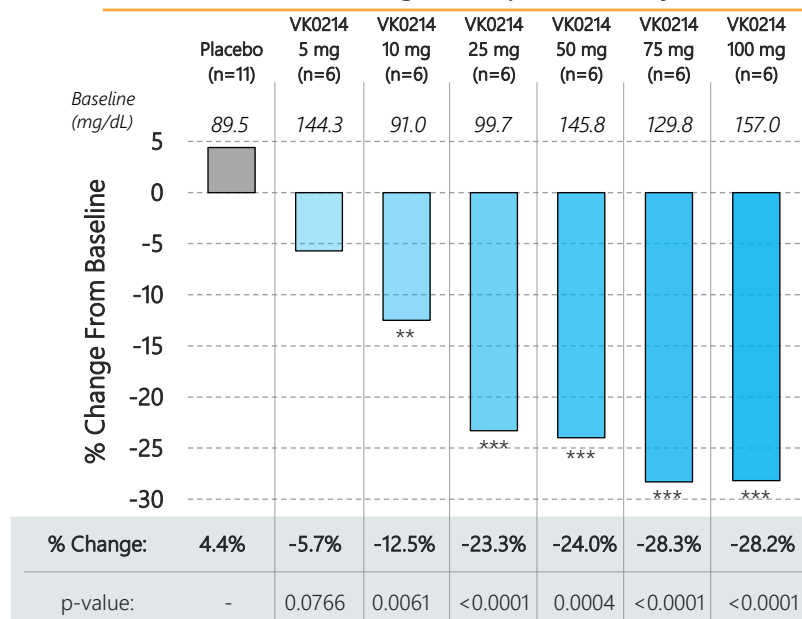
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Overall effect similar to VK2809 12 week experience

VK0214 Apolipoprotein B Effect at 14 Days

- Significant reduction in ApoB observed at all doses >5.0 mg
- Plateau observed at approximately 25% - 30% reduction from baseline
- Lp(a) reductions observed at all doses

Mean % Change in ApoB at Day 14

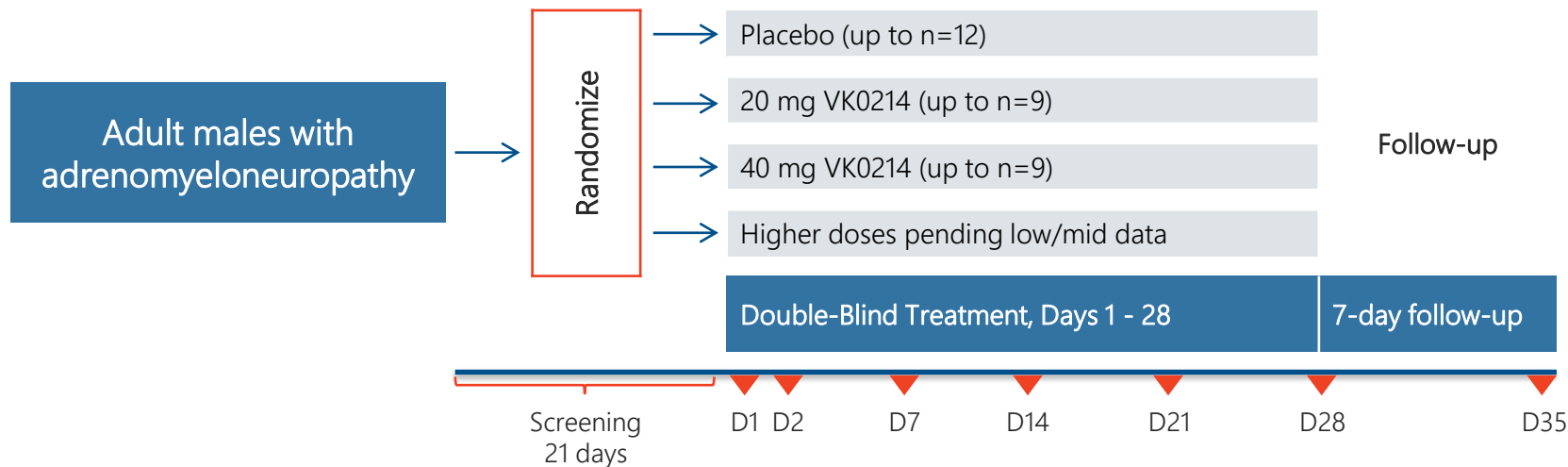


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Takeaways From VK0214 Phase 1 SAD/MAD Study

- Encouraging overall safety and tolerability, no SAEs observed
- No meaningful impact to vital signs, cardiovascular parameters, thyroid axis
- Attractive PK profile; predictable exposures with once-daily dosing
- Preliminary lipid data suggest similar efficacy to VK2809
- Results support further study in adrenomyeloneuropathy (AMN) patients

VK0214 Phase 1b Study in Adrenomyeloneuropathy



- Multicenter, parallel cohort, 28-day Phase 1b trial in adrenomyeloneuropathy
 - Higher doses may be explored pending review of initial cohorts
- Safety, tolerability, change in VLCFAs in male patients with AMN

VK0214 Highlights and Current Status

- Potential to be best in-class oral, small molecule TR β therapeutic for X-ALD
- Encouraging efficacy with rapid (6 weeks) and progressive (up to 25 weeks) VLCFA reductions in plasma, brain, spinal cord and liver
- Phase 1 study in healthy volunteers demonstrated lipid lowering effects
- Phase 1b proof-of-concept study in X-ALD underway
- VK0214 has received Orphan Drug status from the FDA

Financial Summary

- Capital structure and summary financials

Capital Structure ¹	In '000s
Shares outstanding	78,235
Options, RSUs	4,890
Warrants	487
Total shares, options, RSUs, warrants	83,612

Financials	Sept 30, 2021 (\$'000s)
Cash burn YTD 3Q21	\$32,280
Cash and ST Investments	\$216,135

Notes: 1) As of September 30, 2021

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