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Abstract Title : Phase 1 study of ktx-1001, a first-in-class oral MMSET/NSD2 inhibitor, demonstrates clinical activity in relapsed/refractory multiple myeloma

Category: 600s - Hematologic Malignancy

Review Category: Multiple Myeloma: Pharmacologic Therapies

Authors

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Abstract Body

Background

Translocation of chromosomes 4 and 14 (t(4;14)) results in overexpression of MMSET and has been associated with poor clinical outcomes in patients (pts) with multiple myeloma (MM). KTX-1001 is a first-in-class, potent, and selective oral inhibitor of MMSET (also known as NSD2) developed as the first investigational therapy to directly target the high-risk t(4;14) translocation, a key molecular driver in MM. KTX-1001 binds to and inhibits the catalytic SET domain of MMSET, thereby suppressing the deposition of dimethylated histone H3 lysine 36 (H3K36me2), leading to downregulation of oncogenic histone modifications and epigenetic reprogramming of malignant plasma cells. Preclinically, KTX-1001 synergistically inhibited cell viability in MM cell lines when combined with a proteasome inhibitor (PI), immunomodulatory drug (IMiD), or cereblon E3 ligase modulator (CELMoD™). Preliminary findings were previously reported from the initial cohort of 18 pts enrolled in a first-in-human phase 1 trial evaluating KTX-1001 in relapsed/refractory multiple myeloma (RRMM; NCT05651932; Bories et al. *Blood*. 2024;144[Suppl 1]:3370). Here, we present updated results from this study.

Methods

RRMM pts who received ≥3 prior therapies, including PI, IMiD and anti-CD38 monoclonal antibody (anti-CD38 mAb), received oral KTX-1001 across 9 dose levels using a 3+3 dose-escalation design. Primary

objective was to determine the recommended Phase 2 dose (RP2D) and the maximum tolerated dose (MTD). Secondary objectives included pharmacokinetics, pharmacodynamics (PD), and primary efficacy. The dose expansion phase has been initiated to evaluate KTX-1001 in combination with either carfilzomib or the investigational CELMoD™ mezigdomide in t(4;14) RRMM pts.

Results

As of 13 June 2025, 40 pts have been treated in the monotherapy dose-escalation phase. The maximum tolerated dose (MTD) was exceeded due to dose-limiting toxicities (DLTs) in 2 pts: two Grade (Gr) 4 thrombocytopenia and one Gr 3 epistaxis. Backfill at selected dose levels is ongoing to further characterize safety profile and refine dose optimization. The median time from initial MM diagnosis was 8 years (range:2-20) with a median of 6.5 prior lines of therapy (range: 3-25). This included stem cell transplant (70%), BCMA CAR-T (42.5%), and non-CAR-T BCMA targeted drugs such as antibody-drug conjugates and bispecific antibodies (57.5%). The median age was 69 years (range:50-83); 21 were female and 19 were male. Extra-medullary disease was present in 13 (32.5%) pts. Nineteen (47.5%) pts had t(4;14) translocation, 5 (12.5%) had 1q21 gain and 4 (10%) had deletion 17p. A total of 16 (40%) pts have shown a treatment response according to IMWG criteria or have achieved stable disease: 1 VGPR (10+ months), 1 PR (4+ months), 2 MR (3+ and 12 months), 12 SD (2-20+ months). KTX-1001 has demonstrated a favorable tolerability profile. Grade 3 or higher treatment-emergent adverse events (TEAEs) were reported in 31 (77.5%) pts. The most frequent (≥20% pts) Gr 3/4 TEAEs were thrombocytopenia in 12 pts (30%; grade 4 in 8 [20%]), neutropenia in 12 pts (30%; febrile neutropenia in 2 [5%]), anemia in 10 (25%) pts. Gr 3 infections were observed in 5 (12.5%) pts and fatigue in 5 (10%) pts. The main reason for treatment discontinuation was progressive disease (57.5%), other pts discontinued due to physician decision (5%), consent withdrawal (5%), and one pt due to adverse event. Twelve (30%) pts remain on treatment. At Cycle 1 Day 15, drug exposure and steady-state concentrations of KTX-1001 increased with dose. A dose-dependent reduction in H3K36me2, the primary PD marker of KTX-1001 activity, was observed in peripheral blood at Cycle 2 Day 1 and beyond, which plateaued above dose-level 4. Paired bone marrow samples further confirmed on-target PD effects, with a marked reduction in H3K36me2 levels in MM cells at Cycle 2 Day 1 with observed anti-MM effect of reduced proliferation of MM cells with concomitant increased proliferation in immune cells in pts achieving clinical benefit.

Conclusions

KTX-1001 is a novel agent showing tolerable safety profile in RRMM and demonstrating promising single agent activity in heavily pretreated, triple class refractory RRMM including those with high-risk features. KTX is currently evaluated in ongoing study in combination with carfilzomib and the investigational CELMoD™ mezigdomide in t(4;14) RRMM pts.

Keywords: Adult, Treatment Considerations, Clinical Trials, Diseases, Human, Research, Study Population, Clinical Research, Plasma Cell Disorders, Lymphoid Malignancies, Drug Development

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employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Sanofi, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Kite, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Janssen, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, K36 therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Pfizer, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, GSK, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, BMS, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, AstraZeneca, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Forus, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Laura Rosiñol Dachs: Amgen, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Menarini, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, BMS, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Janssen, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the

past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Pfizer, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, GSK, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Sanofi, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Paula Rodriguez-Otero: Johnson and Johnson, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Oncopeptides, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Oncopeptides, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Clinica Universidad de Navarra, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: Yes, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, AstraZeneca, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Innovativev Medicines, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, AbbVie, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Roche, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Menarini Stemline, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Sanofi, Consultancy (Includes expert testimony): Yes,

Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, GSK, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Regeneron, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Pfizer, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, BMS, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Salomon Manier: Regeneron, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Novartis, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Takeda, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Sanofi, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Roche, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Janssen, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Amgen, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, GSK, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Celgene/BMS, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment

in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Adaptive Biotechnology, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Abbvie, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Jesús Berdeja: Pfizer, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Astra Zeneca, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Ichnos Sciences, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Sanofi, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Genentech, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, GSK, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Cartesian Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Kite, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Galapagos, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, BMS, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Karyopharm, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended

employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Roche, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Regeneron, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Karyopharm, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Moderna, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, C4 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Sebi, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Juno Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, J&J, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, CARsgen, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Gracell, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Caribou Biosciences, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Kyowa Kirin, Consultancy (Includes expert

testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Aimaz Afrough: BMS, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Sanofi, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Pfizer, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Johnson & Johnson, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Karyopharm, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Abbvie, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Adaptive Biotechnologies, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Regeneron, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Warren Baker: None declared, Seyed Alireza Hasheminasab: None declared, Anjan Thakurta: K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Vinidhra Sridharan: BMS, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: Yes, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: Yes, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment:

Yes, Current holder of stock options in a privately-held company: Yes, Current equity holder in private company: Yes, Honoraria: No, Erin Flynt: K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: Yes, Current holder of stock options in a privately-held company: Yes, Current equity holder in private company: Yes, Honoraria: No, BMS, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: Yes, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: Yes, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Miriam Barnett: Myeloid Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: Yes, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: Yes, Current equity holder in private company: Yes, Honoraria: No, K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: Yes, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Laura Versmee: K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: Yes, Current holder of stock options in a privately-held company: Yes, Current equity holder in private company: No, Honoraria: No, Takeda, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: Yes, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: Yes, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Daiichi-Sankyo, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: Yes, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Maria-Victoria Mateos: Sanofi, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Johnson and Johnson, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Pfizer, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, BMS/Celgene, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, AstraZeneca, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Stemline, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24

months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Kite, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Amgen, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, AbbVie, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, GlaxoSmithKline, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Cyrille Touzeau: Abbvie, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, J&J, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, GSK, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Amgen, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Pfizer, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Sanofi, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Bristol Myers Squibb, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: No, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: Yes, Alfred Chung: AbbVie, Caelum, CARsgen, K36 Therapeutics, Consultancy (Includes expert testimony): No, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Johnson &Johnson, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research

Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, BMS, Consultancy (Includes expert testimony): Yes, Patents & Royalties: No, Ended employment in the past 24 months: No, Research Funding: Yes, Divested equity in a private or publicly-traded company in the past 24 months: No, Current equity holder in publicly-traded company: No, Current Employment: No, Current holder of stock options in a privately-held company: No, Current equity holder in private company: No, Honoraria: No, Vivek Roy: None declared, Edward Stadtmauer: None declared