

EXHIBIT 5

In the Matter Of:

GVHD KOL Panel - Treatment Practices

Video Recording

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6 The following is a video recording
7 of GVHD KOL Panel - Treatment Practices and
8 Commercial Applications for Novel
9 Therapeutics on June 8, 2020, transcribed by
10 Susan Arnold Yoder, RPR, CRR.
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<p>2</p> <p>1 MS. MERLE: Well, thanks, 2 everyone, for joining us. I'm Ellie Merle. 3 I'm one of the small and mid cap biotech 4 analysts here at Cantor covering Kadmon. 5 I'm very happy to have Dr. Gutman and 6 Dr. Ferrara with us to help us better 7 understand the GVHD landscape. 8 Also on the line are my 9 colleagues Alethia Young, who covers 10 large-cap biotech, and Louise Chen, who 11 covers specialty pharma and large-cap 12 pharma as well. So thank you, guys, for 13 joining us. 14 And before we get started, I 15 just have a quick disclaimer statement to 16 read. The specialists have confirmed that 17 they will not disclose any material 18 nonpublic information. The audience and we 19 will frame our questions appropriately. 20 Also, just a logistical point, 21 there is a Q and A open in the Zoom chat. 22 If you guys have questions, feel free to 23 put those there. 24 And then just before we jump</p>	<p>4</p> <p>1 sort of, you know, how you manage patients 2 your view on the unmet need. 3 DR. FERRARA: Who starts? 4 Should I start? 5 MS. MERLE: Yeah. Go ahead. 6 DR. FERRARA: So I probably 7 myself treat between 20 and 30 patients 8 with acute GVHD and 30-plus patients with 9 chronic GVHD each year. We have an active 10 allogeneic transplant program here at Mount 11 Sinai. 12 But, in addition, I monitor a 13 large consortium that is focused on the 14 natural history of acute GVHD that now 15 includes chronic GVHD. And I've been 16 active in research for new therapies for 17 several decades now. 18 And so I think I have a pretty 19 good view of what the issues are, where the 20 clinical trials are, and what the future is 21 going to be in this therapeutic space. 22 MS. MERLE: Awesome. 23 Dr. Gutman are you there with 24 us? Can you tell us a little bit about</p>
<p>3</p> <p>1 in, I'll just give a quick intro on the 2 biographies of our two speakers who have 3 graciously agreed to speak with us today. 4 So on the line we have 5 Dr. Ferrara, who is currently a professor 6 of hematology and medical oncology at the 7 Mount Sinai Hospital. He's a 8 physician-scientist whose clinical and 9 research careers focus on the immunology of 10 bone marrow transplant, particularly in 11 graph-versus-host disease. 12 Also on the line with us is 13 Jonathan Gutman. He is the director of the 14 allogeneic transplant center at the 15 University of Colorado. He's also served 16 as a co-medical director of the University 17 of Colorado Cord Blood bank. 18 So thank you, guys, very much 19 for joining us today. And I guess, you 20 know, just to kick it off, could you both 21 tell us a bit about your practice in 22 graph-versus-host disease, both in terms of 23 the number of acute and chronic patients 24 that you have, and then we'll jump into</p>	<p>5</p> <p>1 your practice and experience? 2 DR. GUTMAN: Sure. I'm here. 3 And I apologize. I'm having a little video 4 difficulty, but hopefully my voice can 5 stand in and I can send you picture. 6 But I'm an allogeneic stem cell 7 transplant physician also. And our program 8 is an adult transplant program, though we 9 do work with our pediatric colleagues down 10 the street, and I think I have at least a 11 little flavor for what they're up to also. 12 But we do approximately 85 13 allogeneic transplants a year, and I 14 actually end up managing, at least at the 15 top level, the majority of them. 16 So in a typical year I'm 17 probably seeing on the order of 40 or so 18 acute GVHD patients with grade 2 or higher 19 acute GVHD and then follow many chronic 20 GVHD patients also, though we do a lot in 21 our program to try to even avoid chronic 22 GVHD, which is, I'm sure, a topic we could 23 discuss. So that's a little bit about me. 24 MS. MERLE: Great. Thanks.</p>



<p>6</p> <p>1 And then, yeah, I guess jumping 2 into sort of how you manage these patients, 3 both on the acute and chronic side, you 4 know, what proportion respond to steroids, 5 what proportion don't, are there any trends 6 in how this is changing over time, and I 7 guess how you manage patients when they 8 don't respond to steroids. 9 DR. FERRARA: Okay. Well, very 10 briefly, I would say that approximately a 11 little fewer -- a little less than half of 12 the patients respond -- so this is acute 13 GVHD, and you sort of have to separate 14 them. 15 I would say acute GVHD, less 16 than half of the patients respond 17 completely to steroids and need some other 18 form of treatment. You know, we -- GI GVHD 19 is the most worrisome target organ. That's 20 the one that's most resistant to treatment. 21 If someone has GI disease early, we 22 intensify treatment early. 23 We do a number of clinical 24 trials here, and we've done a lot of work</p>	<p>8</p> <p>1 and sometimes the machine isn't available. 2 So that is something of an issue with ECP. 3 But that's, in general, very 4 quickly, how I manage it. I'm sure we'll 5 go into some greater detail a little later 6 on. 7 MS. MERLE: Great. And 8 Dr. Gutman. 9 DR. GUTMAN: So I -- sure. 10 Sure. I think -- I generally agree with 11 Dr. Ferrara, and he's certainly a giant in 12 the field of GVHD. 13 I think a couple little points 14 that I would make, number one for me, 15 again, I think always the first step in 16 trying to deal with GVHD is certainly 17 trying to avoid it up front. 18 And I think some changes in 19 prophylaxis are influencing that, at least, 20 certainly as Dr. Ferrara mentioned, with 21 respect to chronic GVHD, which I do think 22 has implications for future thoughts. 23 With respect to acute 24 graft-versus-host disease, I think one of</p>
<p>7</p> <p>1 with biomarkers that will predict outcomes 2 early on so we can tell which patients are 3 going to be -- get into trouble or not. So 4 we run a lot of GVHD through clinical 5 trials for acute GVHD. 6 Chronic GVHD there are fewer 7 clinical trials at the moment. I would say 8 less than half of the patients respond 9 completely to steroids. Chronic GVHD is 10 starting to be a little less prevalent now 11 that people are using posttransplant 12 Cytoxan as part of the GVHD prophylaxis 13 regimen, but it's still occurring. 14 And some of the patients I use 15 with ibrutinib -- I use ibrutinib for. 16 Others I use ruxolitinib for if there isn't 17 a trial that is ongoing. And sometimes one 18 of my -- in my practice I like 19 extracorporeal photopheresis, particularly 20 for patients with skin disease. 21 The problem with ECP is that 22 the machines are often a great limiting 23 step. You've got to have patients come 24 several times, two to three times a week,</p>	<p>9</p> <p>1 the great challenges with GVHD, which I'm 2 sure we'll explore also, and makes, I 3 think, talking about it difficult is I 4 think that there are a lot of -- there are 5 a lot of things about it that have a degree 6 of subjectivity to it. 7 And though we're working hard, 8 I think, to be as standardized as we can 9 about how we speak about it, I think 10 different folks can have slightly different 11 takes on things. 12 With respect to acute GVHD, I 13 think that -- I believe in my experience in 14 the way we approach this we see a lot of 15 what we would call acute grade 2A or grade 16 2 GVHD, which is really sort of a 17 nausea/vomiting picture that may or may not 18 be called acute GVHD by everybody, may or 19 may not be biopsied by everybody, but I 20 think that's the most common presentation 21 that we see. 22 And because we're maybe quicker 23 to call it -- to call acute grade 2 GVHD 24 than some might be, I think I would say</p>

<p>10</p> <p>1 that all comers we see more responsiveness 2 to steroids. Whether we're really treating 3 a GVHD picture or not, not totally sure. 4 But I would say it's more on 5 the order of 60 to 70 percent of our 6 patients who the majority of whom are this 7 sort of grade 2 that respond quickly to 8 steroids, and we give them a taper of drugs 9 called beclomethasone or budesonide, and 10 they do quite well. 11 But then maybe on the order of 12 30 percent or so to 40 percent, especially 13 now that we have some more agents to use, I 14 would say are a little more steroid 15 refractory. 16 And for that population I think 17 for the acute population ruxolitinib and/or 18 photopheresis are our primary go-to agents 19 with considerations of the details of the 20 patients. 21 And then again with respect to 22 chronic GVHD, here again what's a bit 23 unique about our center is our emphasis on 24 cord blood transplantation, which we do</p>	<p>12</p> <p>1 have -- may look a little different. 2 And particularly after cord 3 blood transplant, as Dr. Gutman says, you 4 get this kind of gray zone. You're not 5 really sure whether it's GVHD or not most 6 of the time, particularly after cord blood 7 transplant because the symptoms are perhaps 8 a little less crisply defined, and you wind 9 up having a different sense of what 10 actually counts as grade 2 or greater. 11 Having said that, we do very 12 few cord blood transplants here. And in 13 fact in general I would say that the number 14 of cord blood transplants is decreasing 15 when you look at big databases like the 16 Center for International Bone Marrow 17 Transplant Research or the National Marrow 18 Donor Program. You know, the number of 19 haploidentical transplants and the use of 20 posttransplant cyclophosphamide has 21 increased overall. 22 So I completely agree with 23 Dr. Gutman, but I do think that the cord 24 blood experience may be a little bit sort</p>
<p>11</p> <p>1 primarily to minimize issues with chronic 2 GVHD, but posttransplant cyclophosphamide 3 in other centers is clearly influencing 4 this issue also. 5 And so I would say that all 6 comers at our center, because we do so many 7 cord blood transplants, we probably see 8 only on the order of about 30 percent or so 9 that have chronic GVHD, 30 to 40, and only 10 about 15 to 20 who have a more severe form 11 that doesn't respond fairly well to some 12 steroids. 13 And then again I would say our 14 main go-to agents in the chronic space 15 right now are primarily ruxolitinib and 16 photopheresis, a little less enthusiastic 17 about ibrutinib, but and then a variety of 18 agents that I know we'll talk about today 19 do have some clinical trial stuff going on 20 also. 21 DR. FERRARA: So if I may, I 22 think Dr. Gutman makes some excellent 23 points. Let me just say that overall acute 24 GVHD, particularly in its early phases, may</p>	<p>13</p> <p>1 of skewed to one end. But definitely you 2 would have some reduction in 3 graft-versus-host disease after cord blood 4 transplants. 5 DR. GUTMAN: Yes. And I just 6 add too, I think -- I don't want to pretend 7 that I expect most centers are doing nearly 8 the cord bloods that we are, and we are 9 looking at more and more haploidentical 10 transplants, and I think our experience is 11 a bit different probably than the general 12 population. 13 But I do think I also get the 14 exposure of the general population the way 15 that I can speak about it meaningfully for 16 sure too, so... 17 MS. MERLE: Absolutely. 18 Awesome. Maybe let's focus a little bit, 19 you know, first on the chronic setting. I 20 mean, I know you were both discussing kind 21 of some of the changes and the trends that 22 we're seeing. 23 What do you expect sort of on a 24 go-forward basis in terms of the</p>

<p>14</p> <p>1 presentation and frequency of chronic 2 graft-versus-host just in terms of, you 3 know, the proportion that comes from 4 progression from acute versus the 5 proportion that sort of occurs 6 spontaneously without acute 7 graft-versus-host disease? 8 And then, I guess, in these 9 patients that don't respond to steroids, 10 you know, if you could go into a little bit 11 more detail on how you manage these 12 patients, how you decide whether to use 13 ruxolitinib, ibrutinib and, you know, what, 14 I guess, you know, is top of mind as you're 15 treating these patients. 16 DR. FERRARA: Right. Well, 17 I'll go first. So chronic 18 graft-versus-host disease can be what we 19 call de novo, which means that it never had 20 acute before. 21 It can be quiescent, which 22 means the patient had acute, we treated it 23 with steroids, all of the acute went away, 24 and then at some point months later chronic</p>	<p>16</p> <p>1 That's one of the issues in chronic. 2 There are -- if you look at the 3 guidelines from the National Cancer Center 4 Network where they've actually now got 5 specific hematopoietic cell transplant 6 guidelines, when you look at 7 steroid-resistant chronic, there are 8 something like 17 different -- at least a 9 dozen different medicines. 10 And they say not one of them, 11 including ruxolitinib and ibrutinib, not 12 one of them can be recommended over another 13 generally. It depends on the patient. It 14 depends on their circumstance. 15 And what that says is that 16 there's still a big unmet medical need in 17 chronic GVHD, particularly 18 steroid-resistant chronic GVHD or this 19 progressive GVHD that has not responded 20 completely. So for someone who's -- so 21 let's put that to the side for a second 22 just to say that there's still a big unmet 23 medical need. 24 If someone, for example, has --</p>
<p>15</p> <p>1 develops. 2 And then it can be progressive. 3 So you get acute, you treat the acute, it 4 doesn't respond completely, and then 5 chronic develops on top of the acute. That 6 progressive form of the disease is the most 7 problematic. It's what kills our patients, 8 and it's the least responsive to treatment. 9 So if someone gets progressive 10 chronic, that means -- often those patients 11 are already on steroids, and as soon as -- 12 they may already be on a second agent 13 because they haven't actually responded yet 14 to therapy. So that therapy might actually 15 be ruxolitinib, or it might be ECP, or it 16 might be something else. 17 So -- but if you then develop 18 chronic, you're going to need something in 19 addition. So if a patient is on steroids 20 and ruxolitinib and has not completely 21 responded and now develops chronic, you're 22 not going to use ruxolitinib. You may 23 actually stop the ruxolitinib depending on 24 what happens and switch to another agent.</p>	<p>17</p> <p>1 by the way, the de novo in my experience is 2 the easiest to treat. If they've never had 3 GVHD and now they get some chronic, usually 4 the large majority of those patients 5 actually respond to steroids. And the 6 quiescent, it's maybe a half, maybe a 7 little less than that. 8 It depends on what -- there are 9 two kinds of problems in chronic. There 10 are the inflammatory changes, so you get 11 inflammatory changes in the mouth or in the 12 skin, and then there are the fibrotic 13 changes where you get sclerodermatous skin 14 and when you get lung disease. 15 Those fibrotic changes are the 16 ones that are the most devastating, that 17 are the most difficult to treat, and where 18 we want -- where we're in desperate need of 19 new agents. In my experience ibrutinib is 20 not good for the sclerotic or fibrotic kind 21 of disease. 22 It is maybe a little more than 23 half of the time good for what we call hot 24 and red, you know, a skin rash, a red skin</p>

<p>18</p> <p>1 rash or a mouth that's got some 2 inflammatory changes. But outside of that 3 I have not found it particularly useful. 4 And so, for example, if a 5 patient of mine is starting to get 6 sclerotic skin disease, that's, for 7 example, where I really push to get them 8 extracorporeal photopheresis because I 9 don't think that ibrutinib is going to 10 work. 11 For in terms of ruxolitinib in 12 terms of the chronic GVHD, I do use it, but 13 it's not my first choice. And part of the 14 problem is that GVHD and steroids are very 15 immunosuppressive. These patients have -- 16 are at very high infectious risk. 17 And ruxolitinib is -- can be 18 myelosuppressive. It can actually drop 19 counts, and it can also be 20 immunosuppressive. And so if I'm really 21 worried about a patient who's had, for 22 example, multiple viral infections or 23 bacterial infections, I tend to stay away 24 from ruxolitinib just because I'm worried</p>	<p>20</p> <p>1 refractory group in whom steroids don't 2 work up front, very challenging disease, 3 very clearly a very significant unmet need. 4 And I think we've got a laundry 5 list of agents of probably relatively 6 comparable efficacy, and we're not great at 7 predicting for any given patient who's 8 going to respond and who's not, and so 9 there's a real art to it. 10 But sort of the three main 11 operating principles that I think I try and 12 think about given those realities are, 13 number one, what is the toxicity of any 14 second-line therapy going to be in terms of 15 its potential medical toxicity, number two, 16 what is the financial toxicity to the 17 patient going to look like because that's a 18 major potential issue, and, number three, 19 what are the logistical considerations 20 around the therapy. 21 And so many of our historic 22 agents with respect to medical toxicity, 23 the immunosuppressive quality of them is 24 very profound. Steroids have a laundry</p>
<p>19</p> <p>1 about its immunosuppressive effects. 2 Let me -- I'll stop there and 3 hand it over to Dr. Gutman. 4 DR. GUTMAN: Thanks. I 5 think -- I certainly agree with Dr. Ferrara 6 with respect to sort of the definitions 7 around chronic GVHD and I think the forms 8 of it that might be most difficult to 9 treat. 10 I think in thinking about the 11 approach to chronic GVHD and one way that I 12 like to kind of break it down and think 13 about it, really the paradigm I think for 14 any GVHD is largely (unintelligible) at 15 this point but particularly I think with 16 respect to chronic the first-line treatment 17 tends to be steroids. 18 And I think we might be making 19 inroads now into maybe not having 20 first-line treatment be steroids or be 21 steroids plus something because steroids 22 are so toxic. 23 But for that portion of 24 patients the traditional steroid-resistant</p>	<p>21</p> <p>1 list of issues. But the immunosuppressive 2 quality of many second-line therapies, as 3 Dr. Ferrara mentioned, is a major concern 4 in these very vulnerable patients. 5 With respect to the financial 6 issues, enormous heterogeneity in our 7 healthcare system about access to these 8 potentially very expensive medications, and 9 that can be very, very variable depending 10 on the details of a particular patient's 11 insurance and impacts access to these 12 medicines. 13 And then in terms of logistics, 14 as Dr. Ferrara also mentioned, I think many 15 of us like ECP for its relatively favorable 16 medical toxicity profile, but its logistics 17 are challenging because it has to be done 18 at a center that can do it. And in a place 19 like Colorado we have a very large 20 catchment area and it can't be done 21 anywhere besides Denver. That can be a 22 major issue. Some medications can be IV 23 versus oral, which can have some impact. 24 But those are factors that I</p>

<p>22</p> <p>1 think are very important to be considering 2 in the context of evaluating any new 3 therapy and how I think about things for 4 any individual patient, and that might 5 impact, you know, the decision between rux, 6 ibrutinib, ECP, or some other agent. 7 MS. MERLE: Got it. That was 8 very helpful. And just real quick before 9 we go further, just a logistical question. 10 Can you remind us what proportion of your 11 chronic GVHD cases are de novo/quiescent 12 versus progressive? 13 DR. FERRARA: Oh, boy. 14 That's -- that's a little hard to say, but 15 I'm going to say 20 percent are 16 progressive, 30 to a third of them are -- 17 maybe 40 percent even are quiescent, so 18 they've responded and now it's come back, 19 and then about 30 percent are or a third 20 are de novo. 21 And the reason I'm hesitating 22 there is sometimes the progressive versus 23 the quiescent, you know, we're tapering 24 steroids because we want to get rid of the</p>	<p>24</p> <p>1 chronic if it was after 100 days. 2 And I think we have recognized 3 now that there's probably pathophysiology 4 rather than time frame that should be 5 defining this, although we're still not 6 great at it. 7 And then there's this entity 8 also of late acute graft-versus-host 9 disease that sort of has characteristics 10 that are more consistent with acute and not 11 chronic but can develop late after the 12 transplant. 13 And whether it behaves exactly 14 like acute or maybe has a little chronic to 15 it or there's some overlap, it's a point 16 that I just put out there as being out 17 there in the ether of consideration, but 18 there are a portion of patients that I 19 think fit into that box too. 20 But overall with respect to 21 quiescent, de novo, I think the numbers 22 Dr. Ferrara quotes are probably similar to 23 what I see. 24 DR. FERRARA: Yes. And just</p>
<p>23</p> <p>1 steroids, and if you taper and then it 2 comes and you've had a complete response 3 and now you've started with chronic, is 4 that actually quiescent or is it 5 progressive because they wind up with 6 steroid-dependent disease. 7 So that's why I was a little -- 8 hesitating a little bit there. But, you 9 know, it's something in that proportion. 10 MS. MERLE: Got it. That's 11 helpful. 12 Dr. Gutman, is this consistent 13 with what you see? 14 DR. GUTMAN: Yeah, I would 15 agree. I think one other point that could 16 be made also, though, is there's also an 17 entity that, again, I think when we get 18 into the reality of looking at these 19 patients can be a little bit of a 20 challenging one to perhaps define. 21 But I think -- historically 22 speaking graft-versus-host disease was 23 characterized historically as acute if it 24 was within 100 days of the transplant and</p>	<p>25</p> <p>1 since it's a new term, this overlap, which 2 is acute and then chronic and then it's 3 usually before day 100, I kind of think of 4 that as early, myself, biologically as 5 early progressive. 6 And in my own practice I'm 7 fairly aggressive in treating that because 8 it means that the immune system control is 9 poor. 10 MS. MERLE: That's helpful. I 11 mean, I think it's interesting from sort of 12 a severity perspective the, you know, 13 high-unmet-need refractory chronic GVHD 14 populations seems to be largely coming from 15 patients, if I'm interpreting your comments 16 correctly, that have already had acute. 17 So if we're looking at, like, 18 front line versus second line, it seems 19 like by the time most of the patients are 20 presenting as severe chronic versus 21 graft-versus-host, they've already had 22 multiple lines of therapy, so in reality 23 it's second line plus, but really it's 24 functionally the front line.</p>

<p>26</p> <p>1 I see Dr. Ferrara nodding, so I 2 assume I didn't get that wrong. 3 So I guess I want to talk about 4 treatment in that setting where you're like 5 the guidelines have 17 different 6 recommendations, there's no clear 7 definition of what should be used. 8 You know, curious your 9 perspective on treating that population and 10 in particular your thoughts on the recent 11 KD025 data and how you think it will be 12 used in the patient population and how 13 you'll decide between KD025, ruxolitinib, 14 ibrutinib, and, I guess, what you think 15 will happen assuming it becomes FDA 16 approved. 17 DR. FERRARA: Well, I would be 18 astonished if it's not FDA approved. 19 MS. MERLE: I would too. 20 DR. FERRARA: There's every 21 indication that it will be. We've been 22 hearing about this now actually for a 23 couple of years. 24 And one of the things that you</p>	<p>28</p> <p>1 maximal response for at least several 2 months. 3 Sometimes, you know, you can 4 get, particularly in a chronic disease, get 5 an improvement for a short period of time. 6 And short here might mean a month or six 7 weeks. But is it still working four months 8 later, five months later, six months later? 9 And at least half of the patients, as I 10 remember, had a response for at least four 11 to five months. So that was good. 12 You didn't see any overt 13 toxicities. This is a pretty fragile 14 population to start with, so to have -- you 15 know, if you saw a toxicity signal, that 16 would be a big red flag because they've 17 already, you know, because the baseline is 18 so high. But they didn't see that. 19 And then one of the things that 20 I always ask about is, as you've heard, we 21 want to reduce steroids in our patients 22 because the steroids are so problematic in 23 terms of their long-term toxicities. So 24 how many patients could effectively have</p>
<p>27</p> <p>1 might not have seen, but the accrual to 2 this trial was really quite rapid. There 3 was a lot of enthusiasm out in the 4 transplant community for the mechanism of 5 action for the drug. 6 And you can often tell -- I 7 think you can, having lived in a clinical 8 trial space, you can tell, particularly in 9 these open label phase twos, you can tell 10 how well something is working by the -- 11 after the first, you know, several months 12 by whether the accrual picks up or not. 13 And this really went up very 14 fast, which meant that both physicians -- 15 physicians felt very comfortable entering 16 their patients onto the clinical trial and 17 treating them with this new agent. And now 18 the top line results look good. They've 19 had high overall response rates, which is, 20 of course, good. 21 The other things that I look 22 for in this -- look for in this trial were 23 the duration of the response, how many 24 patients, you know, did they have this</p>	<p>29</p> <p>1 their steroids reduced? 2 And there was -- I think it was 3 something like about 20 percent could 4 actually have a complete reduction in 5 steroids and 60 percent where there was a 6 60 percent median overall reduction, and so 7 that was very good news. 8 And then, finally, there's a 9 hint that this drug may work for some of 10 that fibrotic disease. So there were at 11 least some partial lung responses that we 12 saw in -- and I call it KD025. 13 (unintelligible). I don't even know how to 14 pronounce the name because I never sort of 15 used the treatment. But, anyway, I think 16 that that's also a very positive sign. 17 MS. MERLE: Got it. And, I 18 mean, I guess, curious as well, Dr. Gutman, 19 but if you could comment on, like, okay, 20 so, you know, if you have this at your 21 fingertips to prescribe to your patients 22 tomorrow, I mean, how would you decide in 23 that proportion seems like kind of anywhere 24 from 25 to 50 percent of the chronic GVHD</p>

<p>30</p> <p>1 that end up getting this sort of 2 progressive hard-to-treat sclerotic type of 3 the disease, how would you decide between 4 rux, ibrutinib, KD025, ECP, and I guess 5 what you think the ultimate proportion will 6 be that get KD025 versus the other 7 treatments.</p> <p>8 DR. GUTMAN: Right. And I 9 would say up front our center has actually 10 not been participating in this trial, so I 11 don't have personal hands-on experience or 12 feel for this drug yet, and so, you know, I 13 think maybe that gives me less bias.</p> <p>14 But I think the data that I'm 15 seeing about it I think is certainly 16 looking very appealing to me in the sense 17 that it's really kind of ticking a lot of 18 those boxes that I had talked about in that 19 the efficacy data looks I would say as good 20 as any GVHD efficacy data that we've seen.</p> <p>21 The side effect toxicity 22 profile looks very, very minimal, and the 23 ease of administration looks very good.</p> <p>24 I mean, the financial issue I</p>	<p>32</p> <p>1 was a phase 3 randomized trial that we 2 could look at, and I think we have to 3 usually start with these -- we very often 4 start with these phase 2 studies or studies 5 where we're kind of looking at investigator 6 assessment of response to a drug.</p> <p>7 But I think the gold standard 8 for really understanding how well things 9 work is generally going to be a randomized 10 trial where we're -- and I think we're now 11 maybe getting to a place in GVHD where we 12 have enough agents that might have efficacy 13 that we could start thinking about that in 14 more meaningful ways.</p> <p>15 But I think there's a long 16 history in GVHD studies of phase 2 studies 17 looking quite promising and when further 18 efforts are undertaken to maybe more 19 robustly or rigorously study the drug, 20 their efficacy may not be quite as high as 21 it appears up front.</p> <p>22 But with that caveat, I think 23 that everything that I've seen about this 24 drug looks very appealing. And to my mind,</p>
<p>31</p> <p>1 can't necessarily speak to, but I will 2 certainly say that an FDA-approved agent is 3 always, I think, on the margins the easier 4 thing to get our hands on than a 5 nonFDA-approved agent in this space where 6 we very often are dealing with drugs that 7 don't have approvals, so that's probably a 8 favorable thing there.</p> <p>9 The one thing that I would sort 10 of say that I think is another universal 11 truth of the history of GVHD and GVHD 12 therapy that I think at least gives me just 13 a grain of salt in thinking about 14 everything is that one of the great 15 challenges that we've had with GVHD therapy 16 for lots of different reasons, including, I 17 think, challenges of just standardization 18 of assessments and logistical issues, et 19 cetera, is that to do very well-conducted 20 trials where we are truly comparing things 21 to get a sense of how well they work has 22 been a very significant issue.</p> <p>23 I mean, I know we got a little 24 information about the itacitinib trial that</p>	<p>33</p> <p>1 I think that it would very quickly be a 2 drug that I would be using as a first-line 3 therapy in steroid-resistant refractory 4 patients just because I think that there 5 are inadequacies in all these other 6 therapies, and I would very quickly be 7 going to it if patients weren't responding 8 to my typical algorithm.</p> <p>9 MS. MERLE: Got it. That's 10 helpful. And I know that, I guess, 11 currently in the chronic GVHD setting, you 12 know, ruxolitinib is not approved, so any 13 use there would be off label.</p> <p>14 But curious, you know, with the 15 REACH3 study ongoing, and I'm sure Alethia, 16 who covers Incyte, has more questions on 17 that as well, but curious your perspective 18 on the ongoing REACH3 study, which, you 19 know, the (unintelligible) for the second 20 half of this year, how this would 21 potentially impact your usage of KD025, 22 assuming it's approved.</p> <p>23 DR. FERRARA: Well, you know, 24 it would have to -- so we've got some</p>

<p>34</p> <p>1 experience with ruxolitinib in chronic, so 2 in some ways it's already known. We would 3 be very interested in, of course, seeing 4 the REACH trial. 5 To my mind, though, it would 6 have to be extremely -- it wouldn't be just 7 a primary end point that was reached. You 8 know, how good is it in the long term? 9 So, for example, in the REACH2 10 study, which has been published, and, now, 11 that was acute graft-versus-host disease, 12 they reached their primary end point. That 13 was fine. But when you look in the 14 supplementary data, six months after the 15 treatment there are no more patients alive 16 who got the ruxolitinib than the other 17 treatments. 18 So they got their response, but 19 then there was some cost, you know, to the 20 treatment or whatever. And what we want at 21 the end of the day is more of our patients 22 alive. 23 So the fact that you use a new 24 treatment, you get a response, but then</p>	<p>36</p> <p>1 for -- well, my firm first choice for any 2 patient who's got steroid-resistant acute 3 is a clinical trial. If there's no 4 clinical trial available, I probably for 5 those patients, I probably use rux -- 6 slightly less than half the time I'll try 7 it. 8 For chronic GVHD patients I, 9 you know, I think it's about -- it's 10 similar. It's maybe -- it's not more than 11 half. It's more than a quarter, but not 12 more than half. 13 MS. YOUNG: Dr. Gutman? 14 DR. GUTMAN: Yeah. I would say 15 we maybe use a little bit more of it. It's 16 probably our first -- in the chronic 17 setting for steroid-resistant refractory 18 it's probably our go-to agent in the 19 majority of patients, maybe 70, 75 percent 20 or so, with the balance primarily being 21 photopheresis and maybe some ibrutinib. 22 On the acute settings I think 23 one of the things that I'm a little 24 concerned about with rux, and I don't know</p>
<p>35</p> <p>1 they die of something else, that doesn't 2 really make you sort of -- doesn't make me 3 rush to that treatment. It says, okay, 4 I've got that somewhere in my, you know, 5 sort of black bag. 6 But since I've already got some 7 experience with rux and since we've seen 8 how that works on the acute side, it would 9 have to be really extremely good to, I 10 think, sort of change people's opinions 11 from what their current practices are. 12 Now, Dr. Gutman makes an 13 excellent point. You know, if it's FDA 14 approved, you're not fighting with the 15 insurance companies, it's not the patients 16 having big out-of-pocket costs, and that is 17 definitely a point in any drug that is FDA 18 approved. 19 MS. YOUNG: Can I just ask you 20 guys both a question about ruxolitinib in 21 general? Can you talk about the percentage 22 of patients that you're kind of using rux 23 in for acute and chronic, please? 24 DR. FERRARA: I would say</p>	<p>37</p> <p>1 if Dr. Ferrera has any thoughts, the worst 2 chronic -- the worst acute GVHD patients 3 are the ones who have very bad gut 4 graft-versus-host disease, and their GI 5 tracts aren't working well at all. 6 And we have a little bit of 7 concern about whether this oral agent is 8 going to be able to be absorbed in the 9 setting of the very sick acute gut 10 graft-versus-host disease patients, and so 11 that gives us a little pause in the patient 12 population in whom I think the issue is 13 most serious. 14 Now, that's a small proportion 15 of our whole for sure. But we may use a 16 little bit more photopheresis in that 17 setting over ruxolitinib. And we still do, 18 although we don't -- we don't have an 19 extraordinary algorithm around it, you 20 know, we do use a little bit of TNF-alpha 21 inhibitor in that population also. 22 And certainly, I mean, it 23 should go without saying that clinical 24 trials are our first choice for these</p>

<p>38</p> <p>1 populations, absolutely. 2 MS. YOUNG: So what percentage 3 of the population have a gut, kind of gut, 4 major severe gut involvement that would 5 restrict you from using ruxolitinib? My 6 sense was like it was, like, under 7 5 percent. Maybe I'm mistaken. 8 DR. FERRARA: Oh, it's higher 9 than that. I would say 10 to 15. 10 MS. YOUNG: Okay. 11 DR. GUTMAN: Yeah. I would put 12 it at about 10 in my -- 13 MS. YOUNG: Okay. About 10. 14 Yeah. Okay. 15 And then I have a question 16 here, I think, from the Q and A. I'll just 17 ask it. And it's if ruxolitinib fails the 18 phase 3 chronic trial versus standard of 19 care, how will you use rux without a label? 20 So you are using rux without a label right 21 now, so kind of interesting. 22 DR. FERRARA: Well, so do 23 you -- if we've got -- if we've got two 24 drugs that have labels and rux doesn't get</p>	<p>40</p> <p>1 about scenarios in which it might be more 2 useful than others, and I think the 3 challenging scenarios are the challenging 4 scenarios. 5 But I also do feel, my personal 6 experience, that ibrutinib has got a little 7 bit more of a toxicity profile associated 8 with it in terms of patients just sort of 9 feeling poorly, some nausea, skin stuff. 10 We see a lot of heart stuff with it, atrial 11 fibrillation, more than I think I might 12 have expected. 13 But certainly if REACH3 looks 14 terrible, I think it might be hard on 15 ruxolitinib. 16 MS. YOUNG: Yeah. Sorry. Go 17 ahead, Louise, or whoever was talking. 18 MS. CHEN: Oh, that was Ellie. 19 Go ahead. 20 MS. YOUNG: Sorry about that. 21 MS. MERLE: No, go ahead. 22 MS. CHEN: All right, guys. 23 I'm going to hop in there, then, since you 24 guys don't have additional questions for</p>
<p>39</p> <p>1 a label, it's not going to be used at all 2 or very rarely because it's got -- we know 3 the downsides and it doesn't have a big 4 upside. 5 So I think it's going to be -- 6 I would think it's going to be very -- 7 people will be hard-pressed to use it if 8 REACH3 fails. 9 DR. GUTMAN: I think it would 10 obviously be concerning if the trial 11 outcomes didn't look good. I think, again, 12 though, there may be hemming and hawing in 13 discussion of trial design and details as 14 to exactly why results might have come out 15 the way they might have. 16 My personal feeling, again, 17 with ibrutinib versus rux, and, again, this 18 is just sort of, again, personal sentiment, 19 and I -- it's something I sort of heard 20 from others, but I don't talk to tons of 21 people around it, is that I haven't been 22 overwhelmingly impressed with ibrutinib as 23 a GVHD agent. 24 I know Dr. Ferrara was talking</p>	<p>41</p> <p>1 now. 2 So I wanted to ask you about a 3 company called Mesoblast. They have a drug 4 called Ryoncil, and it has shown some good 5 data. And I'm curious if you could run 6 through that data with us and let us know 7 what you think of the prospects of this 8 product if it actually gets approved. 9 They've got a PDUFA date coming up in 10 September. 11 DR. FERRARA: Right. So 12 Ryoncil, which is essentially a mesenchymal 13 stromal cell or a mesenchymal stem cell, 14 you know, in some ways it's very 15 interesting. This idea has been around. 16 It was -- the concept was very popular 17 about ten years ago. 18 There were -- but then there 19 was at least one phase 3 trial that failed. 20 So, you know, it didn't work. So it's got 21 that fairly significant baggage to it. 22 Now, they say that they've made 23 some adjustments, and the data that I've 24 seen, they've now got a -- so in children</p>

<p>42</p> <p>1 it does seem to work, at least in patients 2 who have bad gut disease. So we talked 3 about gut disease before. Bad gut disease 4 is bad, you know, like fatal bad. 5 And in a trial where they had, 6 you know, over 50 patients, they had -- 7 what's impressive is that they had not only 8 a high response rate, which was 70 percent, 9 but they had a very good survival three 10 months later, as well as some of the things 11 that you would expect like tapering 12 steroids, which would be very important. 13 So that phase 2 data looks very 14 good. It has -- it does have the problem 15 that it's phase 2, it's not phase 3. It's 16 got this additional problem that previous 17 phase 3 studies have failed. So, you know, 18 how is this one better? And so you have to 19 take that into account. 20 And then there's just a third 21 issue, which is the mechanism of action of 22 this is -- seems to be multiple. You 23 can't -- this isn't like a drug. It's a 24 cellular therapy. But when -- in the</p>	<p>44</p> <p>1 actually fairly large. 2 So let me stop there. 3 MS. CHEN: Dr. Gutman, did you 4 have any thoughts? 5 DR. GUTMAN: You know, I guess 6 I would say as an adult-trained person, I 7 think this notion of this particular 8 product or a permutation of it and 9 mesenchymal stem cells in general, it's 10 certainly been around for a long time, and 11 I don't think we've sort of seen the home 12 runs emerge yet. 13 And I think that they're, to my 14 mind, again, just sort of with my 15 knowledge, there is a large stigma attached 16 to this agent, I would say on the basis of 17 it failing in a real randomized phase 3 18 trial. 19 Again, there are always the 20 details of trial design, but this was only 21 just published in 2020 in our bone marrow 22 transplant journal, the long-term results 23 of the phase 3 study that included adults 24 and kids, and there were secondary</p>
<p>43</p> <p>1 animal models when they did this, well, 2 where are the cells going and what 3 precisely are they doing? 4 The data weren't particularly 5 clear, so it's a little bit fuzzy around 6 some what we would have called the harder 7 science, and now you've got -- and now 8 you've got this previous phase 3 that 9 didn't work. Now you've got a really 10 strong phase 2, but is that the luck of 11 small numbers? We'll have to -- we'll have 12 to see whether the FDA will approve it. 13 But it's my -- since I trained 14 as a pediatrician and I know a lot of 15 pediatric colleagues, they would certainly 16 use it if it's FDA approved. Let's put it 17 that way. 18 And, you know, pediatric 19 studies are hard to do because there are 20 even fewer patients compared to the adult 21 patients. It's about only 20 percent of 22 transplants are in pediatrics. So but it 23 did -- they did manage to get a 50-patient 24 trial, which for a pediatric population is</p>	<p>45</p> <p>1 therapies that also had to be involved. So 2 there are some issues. 3 But I think it points -- that 4 was a randomized trial where you're really 5 going to get an objective look at the data, 6 not just the interpretation of individual 7 people, and it just, the benefit wasn't 8 there. 9 And I understand through my 10 review of the literature, I think, and from 11 my knowledge of my pediatric colleagues, 12 you know, that for whatever reason that the 13 data has been most positive in that 14 pediatric population with the very bad 15 disease, maybe it's very bad disease, 16 period, but it emerges most strongly in the 17 pediatric population, I think -- as an 18 adult doctor I think adult doctors would be 19 somewhat wary of something that only seems 20 to work in pediatric patients. 21 And I don't know whether that 22 would have anything unique to do with the 23 population or not, but I think the market 24 is obviously considerably larger in the</p>

<p>46</p> <p>1 adult population, and I think it's a 2 therapy that probably has an uphill battle 3 to gain traction in that space, especially 4 considering the alternative options that 5 are out there right now. 6 And just kind of looking 7 through the material that you had sent 8 about it, I think it's kind of odd in 9 presenting a balanced picture of this that, 10 like, there's no discussion of a negative 11 phase 3 trial or anything like that, that 12 it's just sort of it feels like it's 13 putting a lot of positive spin on the 14 pediatric experience. 15 But, again, it's also, it's 16 sort of not -- it's historical controls and 17 comparisons, which is always something to 18 be very wary of, particularly against the 19 backdrop of the alternative emerging 20 therapy. 21 That said, I think that these 22 patients who we do have, who are very sick 23 and who are not responding to options, we 24 are desperate for options, and if there is</p>	<p>48</p> <p>1 that the liver is kind of downstream from 2 the gut, so if you've got bad gut, since 3 the liver is a big filter and since we 4 don't often biopsy it and the measurements 5 are crude, the fact that the gut -- that 6 the livers got better, that they were sick 7 and that they got better when the guts got 8 better doesn't surprise me, and it's 9 just -- it didn't make much of an 10 impression. So that was number one. 11 The patients were sick, but the 12 problem is, if you'll notice, the data that 13 they show for I think it was grades 3 and 4 14 and grades C and D, that paper was 15 published, was published in 2005, and that 16 means that the data of those patients is 17 between 18 and 20 years old. 18 So that's -- I don't know 19 what -- 3 to 4s these days don't have 20 disease that is that bad. You don't see -- 21 all of those patients died. And grade 4 is 22 still bad disease, but it's not 100 percent 23 fatal. So I think that that's another 24 grain of salt to put in there.</p>
<p>47</p> <p>1 an approved agent available, even if it's 2 approved for pediatric patients, I think 3 that with a bad acute GVHD patient, I would 4 probably be looking for it off label before 5 I move to some of the more traditional 6 extremely immunosuppressive therapeutics. 7 But I think overall I'm a 8 little bit dubious about its overall 9 potential. 10 MS. CHEN: Okay. Can I follow 11 up really quickly here? So I do know that 12 in the data they showed good efficacy in 13 gut and liver, and you had mentioned the 14 interesting point on the gut side. What 15 about the liver side? Is that important to 16 you? 17 And then the second question I 18 have was just on the grade of severity that 19 you saw of the patients that were involved 20 in the trials versus other trials that are 21 out there for GVHD. 22 DR. FERRARA: So a couple of 23 things. Isolated liver is vanishingly 24 rare. So the way the physiology works is</p>	<p>49</p> <p>1 And just one final thing. 2 Dr. Gutman's absolutely right. In general 3 GVHD occurs less frequently in children and 4 is more easy to treat in children in 5 general. 6 So when you see something that 7 works in kids, many of my adult, you know, 8 adult physician colleagues will say, you 9 know, there may be something special about 10 kids there and mesenchymal stem cells in 11 kids and their healing properties or the 12 resilience of their guts, whatever. 13 So I think that the -- it is 14 going to have, I think, a little bit more 15 of an uphill climb, even if it is approved 16 for children. 17 MS. CHEN: Dr. Gutman, did you 18 have anything to add? 19 DR. GUTMAN: No. I agree with 20 all those things. I would say too, though, 21 with respect to acute graft-versus-host 22 disease of the liver, again, in my 23 experience it just in this day and age 24 doesn't tend to be a tremendously</p>

<p>50</p> <p>1 significant issue for us. 2 We see a lot of, I think, GVHD, 3 chronic GVHD of the liver manifest by we 4 look at the liver a couple of different 5 ways by what we could call kind of a 6 transaminitis and some sort of an angry 7 liver. 8 But the classic liver acute 9 graft-versus-host disease that I think 10 probably even before my time was a very 11 fundamental issue, we just don't tend to 12 see it very much these days. 13 Whether that's a function of 14 some other things like Ursodiol or Actigall 15 and conditioning regimen stuff that we look 16 at, I just don't consider liver GVHD in my 17 experience, acute liver GVHD to be a 18 prominent issue. 19 MS. CHEN: Okay. One last 20 question from me is just the side effect 21 profile that you saw in peds for the study, 22 what did you think of it? 23 DR. FERRARA: Okay. I mean, I 24 didn't see anything. Those patients are so</p>	<p>52</p> <p>1 prevalence of GVHD given perhaps maybe, you 2 know, what you're seeing in terms of 3 transplant trends and usage of CAR T 4 therapies. 5 DR. FERRARA: Well, so you're 6 speaking to two transplanters. We may be 7 slightly biased here. But my sense of CAR 8 Ts is that they are going to be most useful 9 as a bridge to transplant. 10 You know, there was this 11 initial exuberant enthusiasm for, you know, 12 CAR Ts are going to replace transplant, and 13 then the relapses started happening. And 14 once you get -- and transplanting someone 15 in relapse is not a good idea because those 16 patients have way more toxicity and way 17 more -- way worse survival. 18 So I think that as currently 19 constituted the CAR Ts are an excellent 20 bridge to transplant. You use them, you 21 get a nice, a pretty good remission. You 22 then go to transplant and you cure the 23 patient. Might it be useful for some who 24 could not ever get transplants, you know,</p>
<p>51</p> <p>1 sick that it's really hard to see a 2 separate profile. Again, the baseline is 3 so high. So I wouldn't -- I didn't see 4 anything. I didn't see a red flag for 5 sure. 6 DR. GUTMAN: I agree. And, I 7 mean, I think, you know, a theoretical 8 benefit of this therapy is that it would be 9 one that hopefully wouldn't add a ton of 10 toxicity, although perhaps have some 11 immunosuppressive qualities. But I didn't 12 see anything overwhelmingly concerning to 13 my mind either. 14 MS. CHEN: Okay. I'll turn it 15 over back to my colleagues. 16 MS. YOUNG: Go ahead. 17 MS. MERLE: Just two quick 18 ones, if I may. Just first quickly from 19 the inbox, can you comment on long-term 20 trends in GVHD incidence and prevalence 21 given long-term transplant trends and CAR 22 Ts? So basically what the percent change 23 will be, say, in five years, ten years, in 24 terms of sort of the incidence and</p>	<p>53</p> <p>1 some 80-year-old, potentially? Possibly. 2 But the CAR Ts had their own 3 cytokine release syndromes and there are 4 other toxicities. It's not a walk in the 5 park. So I don't see personally that we're 6 going to really replace transplant with CAR 7 T therapy the way some people were talking 8 about that a couple of years ago. 9 DR. GUTMAN: And so the 10 question -- 11 MS. MERLE: Got it. 12 DR. GUTMAN: Sorry. The 13 question, was it just sort of long-term 14 trends in transplant numbers with respect 15 to the future? 16 MS. MERLE: I guess it's the 17 usage of CAR T and sort of the trends that 18 you're seeing today in your practice. I 19 mean, I guess if you see, you know, just to 20 throw out a number, 100 transplants a year, 21 100 cases of GVHD a year, you know, what do 22 you expect that number to be, say, five 23 years from now given the usage and trends 24 that you're seeing with CAR T.</p>

<p>54</p> <p>1 DR. GUTMAN: Right. Well, I 2 mean, I think also the issue should be 3 extended beyond simply CAR T. 4 I mean, you know, I think most 5 transplanters would agree and certainly our 6 nontransplant colleagues for all the 7 diseases for which we do transplants it 8 would be wonderful to have a much simpler 9 alternative potentially curative therapy. 10 And I do think that I wouldn't 11 just be talking about CAR T because, I 12 mean, right now CAR T is really about kind 13 of pediatric ALL and it has some space in 14 non-Hodgkin's lymphoma, but the vast 15 majority of stem cell -- of allogeneic stem 16 cell transplants that we do are more for 17 myeloid malignancies, and I think that 18 we're not close to a CAR T therapeutic 19 option there that doesn't involve 20 transplant. 21 But concurrently we are 22 certainly working on other therapies to 23 improve outcomes for these diseases, and, 24 you know, for example, at our center we're</p>	<p>56</p> <p>1 will continue to have improvements on all 2 fronts. I think the biggest -- the biggest 3 mover on these issues and individual 4 diseases is going to be the alternative 5 therapeutics, not just CAR T, though, that 6 may alter outcomes because you can see sort 7 of piecemeal in disease after disease after 8 disease for which we do these transplants 9 how little bits of them are being taken off 10 by alternative therapies. 11 So, you know, I think it's hard 12 to say for sure, but I also do think that 13 transplant is going to be around for a 14 while yet for sure. 15 DR. FERRARA: You know, it's a 16 little hard to know given that we're, you 17 know, for a couple months we were in this 18 COVID bubble where, you know, a lot of 19 people wouldn't even come to the hospital. 20 You know, in New York this was a major 21 issue. 22 But I would say overall the 23 numbers have, for transplant, have not 24 decreased. If anything they continue to</p>
<p>55</p> <p>1 very versed on the use of a new pill called 2 venetoclax and the use of AML in 3 conjunction with other therapies and 4 probably extending into MDS. 5 And so we're starting to 6 consider who we transplant and who we don't 7 transplant with those diseases as we 8 consider relative risks and benefits as 9 outcomes and alternatives improve. 10 Against that backdrop, though, 11 of course, we're improving outcomes with 12 transplants and improving 13 transplant-related issues, and I think we 14 are making, you know, again, back to my 15 first maybe original point, I think we're 16 making important steps in, A, avoiding GVHD 17 from ever happening, which would be a 18 marked improvement for -- to overcome 19 probably the biggest hurdle around allo 20 transplant, and, B, you know, Dr. Ferrara 21 has been a big leader around these 22 biomarkers and getting much better tools to 23 help deal with GVHD. 24 So I think that, you know, we</p>	<p>57</p> <p>1 increase at about 3, 2 to 3 percent a year. 2 So, you know, in five years are we going 3 still have sort of an 8 to 10 percent 4 increase of transplant? 5 I mean, I would be very 6 surprised if we didn't continue to see a 7 slow increase in the overall number of 8 transplants, even with some of these 9 additional therapies that tend to make the 10 initial remissions longer and deeper. 11 But because the high-risk 12 malignancies for which we use transplant 13 are genetic diseases in which, you know, 14 these very smart cancer cells mutate and 15 figure out ways to become resistant to 16 therapy, I think that we are actually 17 likely to continue to see a steady, maybe 18 slightly slower, but steady increases in 19 transplant numbers, and therefore in the 20 numbers that get some GVHD, although we'll 21 hopefully be able to treat that toxicity 22 and problem better. 23 MS. MERLE: Got it. That's 24 very helpful. And just a last question</p>

<p>58</p> <p>1 just coming from the audience here. Can 2 you talk a little bit more about what you 3 expect the real-world duration will be for 4 KD025 given there's not a lot of sort of 5 treatment alternatives? 6 And I know you mentioned some 7 aspects of the safety profile such as 8 infection risk. So, you know, I mean, from 9 a, you know, how-many-months-you'll-use-it 10 perspective, any color on, you know, what 11 you anticipate will happen in your practice 12 would be helpful. 13 DR. FERRARA: So it's a chronic 14 disease. A patient who's got -- who's 15 getting steroids who needs a second-line 16 therapy, the first thing I want to do, as 17 long as those toxicities are manageable, 18 the toxicities of the new therapy, is to 19 get rid of the steroids. 20 So I would see someone who's 21 let's say KD025 I would imagine it's going 22 to be a minimum of four to six months, and 23 it could be -- and it could be longer. 24 Many of these therapies when</p>	<p>60</p> <p>1 are going the end up on GVHD therapy of 2 some form or another for years and years 3 and years. 4 And with a lot of these 5 patients if we can achieve stability, and 6 in my experience if we have them on a very 7 small dose of prednisone and maybe a small 8 dose of a calcineurin inhibitor, that might 9 be kind of the best we can do. 10 And so the notion of being able 11 to replace those therapies with something 12 that is much less toxic as sort of the 13 anchor for the longer term, I think it's 14 the route I would be thinking about going. 15 But, you know, I think also 16 it's one of these things where I think we 17 probably have personal practice styles to 18 some degree, and, you know, I want to get 19 my hands on the drug and get some 20 experience to see how it feels. 21 But I think, you know, I would 22 guess that there's a lot of patients who 23 aren't going to have complete responses to 24 their underlying issues and all symptoms</p>
<p>59</p> <p>1 they're well tolerated are, particularly if 2 they're oral therapies, are -- and you can 3 reduce the steroid exposure -- and 4 remember, chronic steroid exposure is not 5 just infections. There's muscle wasting. 6 There's diabetes. There are big bone 7 problems. 8 So if I can really minimize 9 those by keeping somebody on a nonsteroid 10 therapy, I would do so. So I would imagine 11 it's going to be, I would say many months. 12 You know, maybe not longer than a year, but 13 between six months and twelve months, I 14 think that's likely to be the case. 15 MS. MERLE: And Dr. Gutman? 16 DR. GUTMAN: Yes. I would 17 certainly agree. You know, I think, again, 18 the management of these patients is very 19 artful. 20 It is -- you know, you have to 21 look at individual patients, but this is a 22 chronic disease, and the vast majority of 23 patients who end up with this disease who 24 are steroid resistant, steroid refractory,</p>	<p>61</p> <p>1 completely go away, and if you haven't 2 achieved that goal then you're often 3 hard-pressed get them off of that last 4 little bit of immunosuppressive therapy -- 5 or GVHD therapy, and this agent would be 6 sort of the last line to me, from what I've 7 seen. 8 MS. MERLE: Got it. Well, 9 we're a little bit over on time, but I want 10 to thank you guys so much for your 11 perspective and joining us today. It was 12 really helpful to hear your insight. 13 And thank you for everyone on 14 the line for joining, and we look forward 15 to speaking with you all soon. Thanks so 16 much. Have a good day. 17 DR. FERRARA: Thanks. Bye. 18 DR. GUTMAN: Thank you. 19 MS. MERLE: Bye. 20 (End of recording.) 21 22 23 24</p>

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1 State of Delaware)
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5 C E R T I F I C A T E

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7 I, Susan Arnold Yoder, Registered

8 Professional Reporter and Notary Public, do

9 hereby certify that the foregoing record,

10 pages 1 to 61 inclusive, is a transcript of

11 my stenographic notes taken from a video

12 recording.

13 IN WITNESS WHEREOF, I have hereunto

14 set my hand and seal this 17th day of July,

15 2020, at Wilmington.

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SUSAN ARNOLD YODER, RPR, CRR



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<p>18 48:17</p> <hr/>	<p>absolutely 13:17 38:1 49:2</p>	<p>alternatives 55:9 58:5</p>
<hr/> <p>2</p> <hr/>	<p>absorbed 37:8</p>	<p>AML 55:2</p>
<p>2 9:16,23 10:7 12:10 32:4,16 42:13,15 43:10 57:1</p>	<p>access 21:7,11</p>	<p>anchor 60:13</p>
<p>20 11:10 22:15 29:3 43:21 48:17</p>	<p>account 42:19</p>	<p>and/or 10:17</p>
<p>2005 48:15</p>	<p>accrual 27:1,12</p>	<p>angry 50:6</p>
<p>2020 44:21</p>	<p>achieve 60:5</p>	<p>animal 43:1</p>
<p>25 29:24</p>	<p>achieved 61:2</p>	<p>anticipate 58:11</p>
<p>2A 9:15</p> <hr/>	<p>Actigall 50:14</p>	<p>appealing 30:16 32:24</p>
<hr/> <p>3</p> <hr/>	<p>action 27:5 42:21</p>	<p>appears 32:21</p>
<p>3 32:1 38:18 41:19 42:15,17 43:8 44:17,23 46:11 48:13,19 57:1</p>	<p>acute 6:3,12,15 7:5 8:23 9:12,15,18, 23 10:17 11:23 14:4,6,20,22,23 15:3, 5 23:23 24:8,10,14 25:2,16 34:11 35:8,23 36:2,22 37:2,9 47:3 49:21 50:8,17</p>	<p>approach 9:14 19:11</p>
<p>30 10:12 11:8,9 22:16,19</p> <hr/>	<p>add 13:6 49:18 51:9</p>	<p>approvals 31:7</p>
<hr/> <p>4</p> <hr/>	<p>addition 15:19</p>	<p>approve 43:12</p>
<p>4 48:13,21</p>	<p>additional 40:24 42:16 57:9</p>	<p>approved 26:16,18 33:12,22 35:14, 18 41:8 43:16 47:1,2 49:15</p>
<p>40 10:12 11:9 22:17</p>	<p>adjustments 41:23</p>	<p>approximately 6:10</p>
<p>4s 48:19</p> <hr/>	<p>administration 30:23</p>	<p>area 21:20</p>
<hr/> <p>5</p> <hr/>	<p>adult 43:20 45:18 46:1 49:7,8</p>	<p>art 20:9</p>
<p>5 38:7</p>	<p>adult-trained 44:6</p>	<p>artful 59:19</p>
<p>50 29:24 42:6</p>	<p>adults 44:23</p>	<p>aspects 58:7</p>
<p>50-patient 43:23</p> <hr/>	<p>age 49:23</p>	<p>assessment 32:6</p>
<hr/> <p>6</p> <hr/>	<p>agent 15:12,24 22:6 27:17 31:2,5 36:18 37:7 39:23 44:16 47:1 61:5</p>	<p>assessments 31:18</p>
<p>60 10:5 29:5,6</p> <hr/>	<p>agents 10:13,18 11:14,18 17:19 20:5,22 32:12</p>	<p>assume 26:2</p>
<hr/> <p>7</p> <hr/>	<p>aggressive 25:7</p>	<p>assuming 26:15 33:22</p>
<p>70 10:5 36:19 42:8</p>	<p>agree 8:10 12:22 19:5 23:15 49:19 51:6 54:5 59:17</p>	<p>astonished 26:18</p>
	<p>ahead 40:17,19,21 51:16</p>	<p>atrial 40:10</p>
	<p>Alethia 33:15</p>	<p>attached 44:15</p>
	<p>algorithm 33:8 37:19</p>	<p>audience 58:1</p>
		<p>avoid 8:17</p>
		<p>avoiding 55:16</p>
		<p>Awesome 13:18</p> <hr/>
		<hr/> <p>B</p> <hr/>
		<p>back 22:18 51:15 55:14</p>

<p>backdrop 46:19 55:10</p> <p>bacterial 18:23</p> <p>bad 37:3 42:2,3,4 45:14,15 47:3 48:2, 20,22</p> <p>bag 35:5</p> <p>baggage 41:21</p> <p>balance 36:20</p> <p>balanced 46:9</p> <p>baseline 28:17 51:2</p> <p>basically 51:22</p> <p>basis 13:24 44:16</p> <p>battle 46:2</p> <p>beclomethasone 10:9</p> <p>behaves 24:13</p> <p>benefit 45:7 51:8</p> <p>benefits 55:8</p> <p>bias 30:13</p> <p>biased 52:7</p> <p>big 12:15 16:16,22 28:16 35:16 39:3 48:3 55:21 59:6</p> <p>biggest 55:19 56:2</p> <p>biologically 25:4</p> <p>biomarkers 7:1 55:22</p> <p>biopsied 9:19</p> <p>biopsy 48:4</p> <p>bit 10:22 12:24 13:11,18 14:10 23:8, 19 36:15 37:6,16,20 40:7 43:5 47:8 49:14 58:2 61:4,9</p> <p>bits 56:9</p> <p>black 35:5</p> <p>blood 10:24 11:7 12:3,6,12,14,24 13:3</p> <p>bloods 13:8</p> <p>bone 12:16 44:21 59:6</p> <p>box 24:19</p> <p>boxes 30:18</p> <p>boy 22:13</p> <p>break 19:12</p> <p>bridge 52:9,20</p>	<p>briefly 6:10</p> <p>bubble 56:18</p> <p>budesonide 10:9</p> <p>Bye 61:17,19</p> <hr/> <p style="text-align: center;">C</p> <hr/> <p>calcineurin 60:8</p> <p>call 9:15,23 14:19 17:23 29:12 50:5</p> <p>called 9:18 10:9 41:3,4 43:6 55:1</p> <p>cancer 16:3 57:14</p> <p>CAR 51:21 52:3,7,12,19 53:2,6,17,24 54:3,11,12,18 56:5</p> <p>care 38:19</p> <p>case 59:14</p> <p>cases 22:11 53:21</p> <p>catchment 21:20</p> <p>caveat 32:22</p> <p>cell 16:5 41:13 54:15,16</p> <p>cells 43:2 44:9 49:10 57:14</p> <p>cellular 42:24</p> <p>center 10:23 11:6 12:16 16:3 21:18 30:9 54:24</p> <p>centers 11:3 13:7</p> <p>cetera 31:19</p> <p>challenges 9:1 31:15,17</p> <p>challenging 20:2 21:17 23:20 40:3</p> <p>change 35:10 51:22</p> <p>changing 6:6</p> <p>characteristics 24:9</p> <p>characterized 23:23</p> <p>CHEN 40:18,22 44:3 47:10 49:17 50:19 51:14</p> <p>children 41:24 49:3,4,16</p> <p>choice 18:13 36:1 37:24</p> <p>chronic 6:3 7:6,9 8:21 10:22 11:1,9, 14 13:19 14:1,17,24 15:5,10,18,21 16:1,7,17,18 17:3,9 18:12 19:7,11,16 22:11 23:3 24:1,11,14 25:2,13,20 28:4 29:24 33:11 34:1 35:23 36:8,16 37:2 38:18 50:3 58:13 59:4,22</p>	<p>circumstance 16:14</p> <p>classic 50:8</p> <p>clear 26:6 43:5</p> <p>climb 49:15</p> <p>clinical 6:23 7:4,7 11:19 27:7,16 36:3,4 37:23</p> <p>close 54:18</p> <p>colleagues 43:15 45:11 49:8 51:15 54:6</p> <p>color 58:10</p> <p>Colorado 21:19</p> <p>comers 10:1 11:6</p> <p>comfortable 27:15</p> <p>comment 29:19 51:19</p> <p>comments 25:15</p> <p>common 9:20</p> <p>community 27:4</p> <p>companies 35:15</p> <p>company 41:3</p> <p>comparable 20:6</p> <p>compared 43:20</p> <p>comparing 31:20</p> <p>comparisons 46:17</p> <p>complete 23:2 29:4 60:23</p> <p>completely 6:17 7:9 12:22 15:4,20 16:20 61:1</p> <p>concept 41:16</p> <p>concern 21:3 37:7</p> <p>concerned 36:24</p> <p>concurrently 54:21</p> <p>conditioning 50:15</p> <p>conjunction 55:3</p> <p>considerably 45:24</p> <p>consideration 24:17</p> <p>considerations 10:19 20:19</p> <p>consistent 23:12 24:10</p> <p>constituted 52:19</p> <p>context 22:2</p> <p>continue 56:1,24 57:6,17</p>
--	--	---

control 25:8 controls 46:16 cord 10:24 11:7 12:2,6,12,14,23 13:3,8 correctly 25:16 cost 34:19 costs 35:16 counts 12:10 18:19 couple 8:13 26:23 47:22 50:4 53:8 56:17 covers 33:16 COVID 56:18 crisply 12:8 crude 48:5 curative 54:9 cure 52:22 curious 26:8 29:18 33:14,17 41:5 current 35:11 cyclophosphamide 11:2 12:20 cytokine 53:3 Cytosan 7:12	defined 12:8 defining 24:5 definition 26:7 definitions 19:6 degree 9:5 60:18 Denver 21:21 depending 15:23 21:9 depends 16:13,14 17:8 design 39:13 44:20 desperate 17:18 46:24 detail 8:5 14:11 details 10:19 21:10 39:13 44:20 devastating 17:16 develop 15:17 24:11 develops 15:1,5,21 diabetes 59:6 die 35:1 died 48:21 difficult 9:3 17:17 19:8 discussing 13:20 discussion 39:13 46:10 disease 6:21 7:20 8:24 13:3 14:7,18 15:6 17:14,21 18:6 20:2 23:6,22 24:9 28:4 29:10 30:3 34:11 37:4,10 42:2,3 45:15 48:20,22 49:22 50:9 56:7,8 58:14 59:22,23 diseases 54:7,23 55:7 56:4 57:13 doctor 45:18 doctors 45:18 Donor 12:18 dose 60:7,8 downsides 39:3 downstream 48:1 dozen 16:9 drop 18:18 drug 27:5 29:9 30:12 32:6,19,24 33:2 35:17 41:3 42:23 60:19 drugs 10:8 31:6 38:24 dubious 47:8	duration 27:23 58:3
E		
		early 6:21,22 7:2 11:24 25:4,5 ease 30:23 easier 31:3 easiest 17:2 easy 49:4 ECP 7:21 8:2 15:15 21:15 22:6 30:4 effect 30:21 50:20 effectively 28:24 effects 19:1 efficacy 20:6 30:19,20 32:12,20 47:12 efforts 32:18 Ellie 40:18 emerge 44:12 emerges 45:16 emerging 46:19 emphasis 10:23 end 13:1 30:1 34:7,12,21 59:23 60:1 61:20 enormous 21:6 entering 27:15 enthusiasm 27:3 52:11 enthusiastic 11:16 entity 23:17 24:7 essentially 41:12 ether 24:17 evaluating 22:2 excellent 11:22 35:13 52:19 expect 13:7,23 42:11 53:22 58:3 expected 40:12 expensive 21:8 experience 9:13 12:24 13:10 17:1, 19 30:11 34:1 35:7 40:6 46:14 49:23 50:17 60:6,20 explore 9:2 exposure 13:14 59:3,4

<p>extended 54:3</p> <p>extending 55:4</p> <p>extracorporeal 7:19 18:8</p> <p>extraordinary 37:19</p> <p>extremely 34:6 35:9 47:6</p> <p>exuberant 52:11</p> <hr/> <p style="text-align: center;">F</p> <hr/> <p>fact 12:13 34:23 48:5</p> <p>factors 21:24</p> <p>failed 41:19 42:17</p> <p>failing 44:17</p> <p>fails 38:17 39:8</p> <p>fairly 11:11 25:7 41:21 44:1</p> <p>fast 27:14</p> <p>fatal 42:4 48:23</p> <p>favorable 21:15 31:8</p> <p>FDA 26:15,18 35:13,17 43:12,16</p> <p>FDA-APPROVED 31:2</p> <p>feel 30:12 40:5</p> <p>feeling 39:16 40:9</p> <p>feels 46:12 60:20</p> <p>felt 27:15</p> <p>Ferrara 6:9 8:11,20 11:21 14:16 19:5 21:3,14 22:13 24:22,24 26:1,17,20 33:23 35:24 38:8,22 39:24 41:11 47:22 50:23 52:5 55:20 56:15 58:13 61:17</p> <p>Ferrera 37:1</p> <p>fewer 6:11 7:6 43:20</p> <p>fibrillation 40:11</p> <p>fibrotic 17:12,15,20 29:10</p> <p>field 8:12</p> <p>fighting 35:14</p> <p>figure 57:15</p> <p>filter 48:3</p> <p>final 49:1</p> <p>finally 29:8</p> <p>financial 20:16 21:5 30:24</p>	<p>fine 34:13</p> <p>fingertips 29:21</p> <p>firm 36:1</p> <p>first-line 19:16,20 33:2</p> <p>fit 24:19</p> <p>flag 28:16 51:4</p> <p>focus 13:18</p> <p>folks 9:10</p> <p>follow 47:10</p> <p>form 6:18 11:10 15:6 60:2</p> <p>forms 19:7</p> <p>forward 61:14</p> <p>found 18:3</p> <p>fragile 28:13</p> <p>frame 24:4</p> <p>frequency 14:1</p> <p>frequently 49:3</p> <p>front 8:17 20:2 25:18,24 30:9 32:21</p> <p>fronts 56:2</p> <p>function 50:13</p> <p>functionally 25:24</p> <p>fundamental 50:11</p> <p>future 8:22 53:15</p> <p>fuzzy 43:5</p> <hr/> <p style="text-align: center;">G</p> <hr/> <p>gain 46:3</p> <p>general 8:3 12:13 13:11,14 35:21 44:9 49:2,5</p> <p>generally 8:10 16:13 32:9</p> <p>genetic 57:13</p> <p>GI 6:18,21 37:4</p> <p>giant 8:11</p> <p>give 10:8</p> <p>go-forward 13:24</p> <p>go-to 10:18 11:14 36:18</p> <p>goal 61:2</p> <p>gold 32:7</p>	<p>good 17:20,23 27:18,20 28:11 29:7 30:19,23 34:8 35:9 39:11 41:4 42:9,14 47:12 52:15,21 61:16</p> <p>grade 9:15,23 10:7 12:10 47:18 48:21</p> <p>grades 48:13,14</p> <p>graft-versus-host 8:24 13:3 14:2,7,18 23:22 24:8 25:21 34:11 37:4,10 49:21 50:9</p> <p>grain 31:13 48:24</p> <p>gray 12:4</p> <p>great 7:22 8:7 9:1 20:6 24:6 31:14</p> <p>greater 8:5 12:10</p> <p>group 20:1</p> <p>guess 6:1,7 14:8,14 26:3,14 29:18 30:4 33:10 44:5 53:16,19 60:22</p> <p>guidelines 16:3,6 26:5</p> <p>gut 37:3,9 38:3,4 42:2,3 47:13,14 48:2,5</p> <p>Gutman 8:8,9 11:22 12:3,23 13:5 19:3,4 23:12,14 29:18 30:8 35:12 36:13,14 38:11 39:9 44:3,5 49:17,19 51:6 53:9,12 54:1 59:15,16 61:18</p> <p>Gutman's 49:2</p> <p>guts 48:7 49:12</p> <p>guys 35:20 40:22,24 61:10</p> <p>GVHD 6:13,15,18 7:4,5,6,9,12 8:12,16,21 9:1,12,16,18,23 10:3,22 11:2,9,24 12:5 16:17,18,19 17:3 18:12,14 19:7,11,14 22:11 25:13 29:24 30:20 31:11,15 32:11,16 33:11 36:8 37:2 39:23 47:3,21 49:3 50:2,3,16,17 51:20 52:1 53:21 55:16,23 57:20 60:1 61:5</p> <hr/> <p style="text-align: center;">H</p> <hr/> <p>half 6:11,16 7:8 17:6,23 28:9 33:20 36:6,11,12</p> <p>hand 19:3</p> <p>hands 31:4 60:19</p> <p>hands-on 30:11</p> <p>haploidetical 12:19 13:9</p> <p>happen 26:15 58:11</p> <p>happening 52:13 55:17</p>
---	--	---

<p>hard 9:7 22:14 40:14 43:19 51:1 56:11,16</p> <p>hard-pressed 39:7 61:3</p> <p>hard-to-treat 30:2</p> <p>harder 43:6</p> <p>hawing 39:12</p> <p>healing 49:11</p> <p>healthcare 21:7</p> <p>hear 61:12</p> <p>heard 28:20 39:19</p> <p>hearing 26:22</p> <p>heart 40:10</p> <p>helpful 22:8 23:11 25:10 33:10 57:24 58:12 61:12</p> <p>hematopoietic 16:5</p> <p>hemming 39:12</p> <p>hesitating 22:21 23:8</p> <p>heterogeneity 21:6</p> <p>high 18:16 27:19 28:18 32:20 42:8 51:3</p> <p>high-risk 57:11</p> <p>high-unmet-need 25:13</p> <p>higher 38:8</p> <p>hint 29:9</p> <p>historic 20:21</p> <p>historical 46:16</p> <p>historically 23:21,23</p> <p>history 31:11 32:16</p> <p>home 44:11</p> <p>hop 40:23</p> <p>hospital 56:19</p> <p>hot 17:23</p> <p>how-many-months-you'll-use-it 58:9</p> <p>hurdle 55:19</p>	<p>idea 41:15 52:15</p> <p>imagine 58:21 59:10</p> <p>immune 25:8</p> <p>immunosuppressive 18:15,20 19:1 20:23 21:1 47:6 51:11 61:4</p> <p>impact 21:23 22:5 33:21</p> <p>impacts 21:11</p> <p>implications 8:22</p> <p>important 22:1 42:12 47:15 55:16</p> <p>impressed 39:22</p> <p>impression 48:10</p> <p>impressive 42:7</p> <p>improve 54:23 55:9</p> <p>improvement 28:5 55:18</p> <p>improvements 56:1</p> <p>improving 55:11,12</p> <p>inadequacies 33:5</p> <p>inbox 51:19</p> <p>incidence 51:20,24</p> <p>included 44:23</p> <p>including 16:11 31:16</p> <p>increase 57:1,4,7</p> <p>increased 12:21</p> <p>increases 57:18</p> <p>Incyte 33:16</p> <p>indication 26:21</p> <p>individual 22:4 45:6 56:3 59:21</p> <p>infection 58:8</p> <p>infections 18:22,23 59:5</p> <p>infectious 18:16</p> <p>inflammatory 17:10,11 18:2</p> <p>influencing 8:19 11:3</p> <p>information 31:24</p> <p>inhibitor 37:21 60:8</p> <p>initial 52:11 57:10</p> <p>inroads 19:19</p> <p>insight 61:12</p> <p>insurance 21:11 35:15</p>	<p>intensify 6:22</p> <p>interested 34:3</p> <p>interesting 25:11 38:21 41:15 47:14</p> <p>International 12:16</p> <p>interpretation 45:6</p> <p>interpreting 25:15</p> <p>investigator 32:5</p> <p>involve 54:19</p> <p>involved 45:1 47:19</p> <p>involvement 38:4</p> <p>Isolated 47:23</p> <p>issue 8:2 11:4 20:18 21:22 30:24 31:22 37:12 42:21 50:1,11,18 54:2 56:21</p> <p>issues 11:1 16:1 21:1,6 31:18 45:2 55:13 56:3 60:24</p> <p>itacitinib 31:24</p> <p>IV 21:22</p>
J		
<p>joining 61:11,14</p> <p>journal 44:22</p> <p>jumping 6:1</p>		
K		
<p>KD025 26:11,13 29:12 30:4,6 33:21 58:4,21</p> <p>keeping 59:9</p> <p>kids 44:24 49:7,10,11</p> <p>kills 15:7</p> <p>kind 12:4 13:20 17:20 19:12 25:3 29:23 30:17 32:5 35:22 38:3,21 46:6, 8 48:1 50:5 54:12 60:9</p> <p>kinds 17:9</p> <p>knowledge 44:15 45:11</p>		
L		
<p>label 27:9 33:13 38:19,20 39:1 47:4</p> <p>labels 38:24</p>		

<p>large 17:4 21:19 44:1,15</p> <p>largely 19:14 25:14</p> <p>larger 45:24</p> <p>late 24:8,11</p> <p>laundry 20:4,24</p> <p>leader 55:21</p> <p>limiting 7:22</p> <p>lines 25:22</p> <p>list 20:5 21:1</p> <p>literature 45:10</p> <p>lived 27:7</p> <p>liver 47:13,15,23 48:1,3 49:22 50:3,4,7,8,16,17</p> <p>livers 48:6</p> <p>logistical 20:19 22:9 31:18</p> <p>logistics 21:13,16</p> <p>long 32:15 34:8 44:10 58:17</p> <p>long-term 28:23 44:22 51:19,21 53:13</p> <p>longer 57:10 58:23 59:12 60:13</p> <p>lot 6:24 7:4 9:4,5,14 27:3 30:17 40:10 43:14 46:13 50:2 56:18 58:4 60:4,22</p> <p>lots 31:16</p> <p>Louise 40:17</p> <p>luck 43:10</p> <p>lung 17:14 29:11</p> <p>lymphoma 54:14</p> <hr/> <p style="text-align: center;">M</p> <hr/> <p>machine 8:1</p> <p>machines 7:22</p> <p>made 23:16 41:22</p> <p>main 11:14 20:10</p> <p>major 20:18 21:3,22 38:4 56:20</p> <p>majority 10:6 17:4 36:19 54:15 59:22</p> <p>make 8:14 35:2 48:9 57:9</p> <p>makes 9:2 11:22 35:12</p> <p>making 19:18 55:14,16</p>	<p>malignancies 54:17 57:12</p> <p>manage 6:2,7 8:4 14:11 43:23</p> <p>manageable 58:17</p> <p>management 59:18</p> <p>manifest 50:3</p> <p>margins 31:3</p> <p>marked 55:18</p> <p>market 45:23</p> <p>marrow 12:16,17 44:21</p> <p>material 46:7</p> <p>maximal 28:1</p> <p>MDS 55:4</p> <p>meaningful 32:14</p> <p>meaningfully 13:15</p> <p>means 14:19,22 15:10 25:8 48:16</p> <p>meant 27:14</p> <p>measurements 48:4</p> <p>mechanism 27:4 42:21</p> <p>median 29:6</p> <p>medical 16:16,23 20:15,22 21:16</p> <p>medications 21:8,22</p> <p>medicines 16:9 21:12</p> <p>mentioned 8:20 21:3,14 47:13 58:6</p> <p>MERLE 8:7 13:17 22:7 23:10 25:10 26:19 29:17 33:9 40:21 51:17 53:11,16 57:23 59:15 61:8,19</p> <p>mesenchymal 41:12,13 44:9 49:10</p> <p>Mesoblast 41:3</p> <p>mind 14:14 32:24 34:5 44:14 51:13</p> <p>mine 18:5</p> <p>minimal 30:22</p> <p>minimize 11:1 59:8</p> <p>minimum 58:22</p> <p>mistaken 38:7</p> <p>models 43:1</p> <p>moment 7:7</p> <p>month 28:6</p> <p>months 14:24 27:11 28:2,7,8,11 34:14 42:10 56:17 58:22 59:11,13</p>	<p>mouth 17:11 18:1</p> <p>move 47:5</p> <p>mover 56:3</p> <p>multiple 18:22 25:22 42:22</p> <p>muscle 59:5</p> <p>mutate 57:14</p> <p>myeloid 54:17</p> <p>myelosuppressive 18:18</p> <hr/> <p style="text-align: center;">N</p> <hr/> <p>National 12:17 16:3</p> <p>nausea 40:9</p> <p>nausea/vomiting 9:17</p> <p>necessarily 31:1</p> <p>negative 46:10</p> <p>Network 16:4</p> <p>news 29:7</p> <p>nice 52:21</p> <p>nodding 26:1</p> <p>non-hodgkin's 54:14</p> <p>nonfda-approved 31:5</p> <p>nonsteroid 59:9</p> <p>nontransplant 54:6</p> <p>notice 48:12</p> <p>notion 44:7 60:10</p> <p>novo 14:19 17:1 22:20 24:21</p> <p>novo/quiescent 22:11</p> <p>number 6:23 8:14 12:13,18 20:13,15,18 48:10 53:20,22 57:7</p> <p>numbers 24:21 43:11 53:14 56:23 57:19,20</p> <hr/> <p style="text-align: center;">O</p> <hr/> <p>objective 45:5</p> <p>occurring 7:13</p> <p>occurs 14:5 49:3</p> <p>odd 46:8</p> <p>ongoing 7:17 33:15,18</p>
--	---	---

open 27:9
operating 20:11
opinions 35:10
option 54:19
options 46:4,23,24
oral 21:23 37:7 59:2
order 10:5,11 11:8
organ 6:19
original 55:15
out-of-pocket 35:16
outcomes 7:1 39:11 54:23 55:9,11 56:6
overcome 55:18
overlap 24:15 25:1
overt 28:12
overwhelmingly 39:22 51:12

P

paper 48:14
paradigm 19:13
park 53:5
part 7:12 18:13
partial 29:11
participating 30:10
pathophysiology 24:3
patient 14:22 15:19 16:13 18:5,21 20:7,17 22:4 26:12 36:2 37:11 47:3 52:23 58:14
patient's 21:10
patients 6:2,7,12,16 7:2,8,14,20,23 10:6,20 14:9,12,15 15:7,10 17:4 18:15 19:24 21:4 23:19 24:18 25:15, 19 27:16,24 28:9,21,24 29:21 33:4,7 34:15,21 35:15,22 36:5,8,19 37:2,10 40:8 42:1,6 43:20,21 45:20 46:22 47:2,19 48:11,16,21 50:24 52:16 59:18,21,23 60:5,22
pause 37:11
PDUFA 41:9
pediatric 43:15,18,24 45:11,14,17, 20 46:14 47:2 54:13

pediatrician 43:14
pediatrics 43:22
peds 50:21
people 7:11 39:7,21 45:7 53:7 56:19
people's 35:10
percent 10:5,12 11:8 22:15,17,19 29:3,5,6,24 36:19 38:7 42:8 43:21 48:22 51:22 57:1,3
percentage 35:21 38:2
period 28:5 45:16
permutation 44:8
person 44:6
personal 30:11 39:16,18 40:5 60:17
personally 53:5
perspective 25:12 26:9 33:17 58:10 61:11
phase 27:9 32:1,4,16 38:18 41:19 42:13,15,17 43:8,10 44:17,23 46:11
phases 11:24
photopheresis 7:19 10:18 11:16 18:8 36:21 37:16
physician 49:8
physicians 27:14,15
physiology 47:24
picks 27:12
picture 9:17 10:3 46:9
piecemeal 56:7
pill 55:1
place 21:18 32:11
point 14:24 19:15 23:15 24:15 34:7, 12 35:13,17 47:14 55:15
points 8:13 11:23 45:3
poor 25:9
poorly 40:9
popular 41:16
population 10:16,17 13:12,14 26:9, 12 28:14 37:12,21 38:3 43:24 45:14, 17,23 46:1
populations 25:14 38:1
portion 19:23 24:18

positive 29:16 45:13 46:13
Possibly 53:1
posttransplant 7:11 11:2 12:20
potential 20:15,18 47:9
potentially 21:8 33:21 53:1 54:9
practice 7:18 25:6 53:18 58:11 60:17
practices 35:11
precisely 43:3
predict 7:1
predicting 20:7
prednisone 60:7
prescribe 29:21
presentation 9:20 14:1
presenting 25:20 46:9
pretend 13:6
pretty 28:13 52:21
prevalence 51:20 52:1
prevalent 7:10
previous 42:16 43:8
primarily 11:1,15 36:20
primary 10:18 34:7,12
principles 20:11
problem 7:21 18:14 42:14,16 48:12 57:22
problematic 15:7 28:22
problems 17:9 59:7
product 41:8 44:8
profile 21:16 30:22 40:7 50:21 51:2 58:7
profound 20:24
Program 12:18
progression 14:4
progressive 15:2,6,9 16:19 22:12, 16,22 23:5 25:5 30:2
prominent 50:18
promising 32:17
pronounce 29:14
properties 49:11
prophylaxis 7:12 8:19

proportion 6:4,5 14:3,5 22:10 23:9
29:23 30:5 37:14

prospects 41:7

published 34:10 44:21 48:15

push 18:7

put 16:21 24:16 38:11 43:16 48:24

putting 46:13

Q

qualities 51:11

quality 20:23 21:2

quarter 36:11

question 22:9 35:20 38:15 47:17
50:20 53:10,13 57:24

questions 33:16 40:24

quick 22:8 51:17

quicker 9:22

quickly 8:4 10:7 33:1,6 47:11 51:18

quiescent 14:21 17:6 22:17,23 23:4
24:21

quotes 24:22

R

randomized 32:1,9 44:17 45:4

rapid 27:2

rare 47:24

rarely 39:2

rash 17:24 18:1

rate 42:8

rates 27:19

REACH 34:4

REACH2 34:9

REACH3 33:15,18 39:8 40:13

reached 34:7,12

real 20:9 22:8 44:17

real-world 58:3

realities 20:12

reality 23:18 25:22

reason 22:21 45:12

reasons 31:16

recent 26:10

recognized 24:2

recommendations 26:6

recommended 16:12

recording 61:20

red 17:24 28:16 51:4

reduce 28:21 59:3

reduced 29:1

reduction 13:2 29:4,6

refractory 10:15 20:1 25:13 33:3
36:17 59:24

regimen 7:13 50:15

relapse 52:15

relapses 52:13

relative 55:8

release 53:3

remember 28:10 59:4

remind 22:10

remission 52:21

remissions 57:10

replace 52:12 53:6 60:11

Research 12:17

resilience 49:12

resistent 6:20 57:15 59:24

respect 8:21,23 9:12 10:21 19:6,16
20:22 21:5 24:20 49:21 53:14

respond 6:4,8,12,16 7:8 10:7 11:11
14:9 15:4 17:5 20:8

responded 15:13,21 16:19 22:18

responding 33:7 46:23

response 23:2 27:19,23 28:1,10
32:6 34:18,24 42:8

responses 29:11 60:23

responsive 15:8

responsiveness 10:1

restrict 38:5

results 27:18 39:14 44:22

review 45:10

rid 22:24 58:19

rigorously 32:19

risk 18:16 58:8

risks 55:8

robustly 32:19

route 60:14

run 7:4 41:5

runs 44:12

rush 35:3

ruX 22:5 30:4 35:7,22 36:5,24 38:19,
20,24 39:17

ruxolitinib 7:16 10:17 11:15 14:13
15:15,20,22,23 16:11 18:11,17,24
26:13 33:12 34:1,16 35:20 37:17
38:5,17 40:15

Ryoncil 41:4,12

S

safety 58:7

salt 31:13 48:24

scenarios 40:1,3,4

science 43:7

sclerodermatous 17:13

sclerotic 17:20 18:6 30:2

second-line 20:14 21:2 58:15

secondary 44:24

sense 12:9 30:16 31:21 38:6 52:7

sentiment 39:18

separate 6:13 51:2

September 41:10

setting 13:19 26:4 33:11 36:17 37:9,
17

settings 36:22

severe 11:10 25:20 38:4

severity 25:12 47:18

short 28:5,6

show 48:13

showed 47:12

<p>shown 41:4</p> <p>sick 37:9 46:22 48:6,11 51:1</p> <p>side 6:3 16:21 30:21 35:8 47:14,15 50:20</p> <p>sign 29:16</p> <p>signal 28:15</p> <p>significant 20:3 31:22 41:21 50:1</p> <p>similar 24:22 36:10</p> <p>simpler 54:8</p> <p>simply 54:3</p> <p>skewed 13:1</p> <p>skin 7:20 17:12,13,24 18:6 40:9</p> <p>slightly 9:10 36:6 52:7 57:18</p> <p>slow 57:7</p> <p>slower 57:18</p> <p>small 37:14 43:11 60:7</p> <p>smart 57:14</p> <p>sort 6:2,13 9:16 10:7 12:24 13:23 14:5 19:6 20:10 24:9 25:11 29:14 30:1 31:9 35:2,5,10 39:18,19 40:8 44:11,14 46:12,16 50:6 51:24 53:13, 17 56:6 57:3 58:4 60:12 61:6</p> <p>space 11:14 27:8 31:5 46:3 54:13</p> <p>speak 9:9 13:15 31:1</p> <p>speaking 23:22 52:6 61:15</p> <p>special 49:9</p> <p>specific 16:5</p> <p>spin 46:13</p> <p>spontaneously 14:6</p> <p>stability 60:5</p> <p>standard 32:7 38:18</p> <p>standardization 31:17</p> <p>standardized 9:8</p> <p>start 28:14 32:3,4,13</p> <p>started 23:3 52:13</p> <p>starting 7:10 18:5 55:5</p> <p>stay 18:23</p> <p>steady 57:17,18</p> <p>stem 41:13 44:9 49:10 54:15</p>	<p>step 7:23 8:15</p> <p>steps 55:16</p> <p>steroid 10:14 59:3,4,24</p> <p>steroid-dependent 23:6</p> <p>steroid-resistant 16:7,18 19:24 33:3 36:2,17</p> <p>steroids 6:4,8,17 7:9 10:2,8 11:12 14:9,23 15:11,19 17:5 18:14 19:17, 20,21 20:1,24 22:24 23:1 28:21,22 29:1,5 42:12 58:15,19</p> <p>stigma 44:15</p> <p>stop 15:23 19:2 44:2</p> <p>stromal 41:13</p> <p>strong 43:10</p> <p>strongly 45:16</p> <p>studies 32:4,16 42:17 43:19</p> <p>study 32:19 33:15,18 34:10 44:23 50:21</p> <p>stuff 11:19 40:9,10 50:15</p> <p>styles 60:17</p> <p>subjectivity 9:6</p> <p>supplementary 34:14</p> <p>surprise 48:8</p> <p>surprised 57:6</p> <p>survival 42:9 52:17</p> <p>switch 15:24</p> <p>symptoms 12:7 60:24</p> <p>syndromes 53:3</p> <p>system 21:7 25:8</p>	<p>tend 18:23 49:24 50:11 57:9</p> <p>term 25:1 34:8 60:13</p> <p>terms 13:24 14:2 18:11,12 20:14 21:13 28:23 40:8 51:24 52:2</p> <p>terrible 40:14</p> <p>theoretical 51:7</p> <p>therapeutic 54:18</p> <p>therapeutics 47:6 56:5</p> <p>therapies 21:2 33:6 45:1 52:4 54:22 55:3 56:10 57:9 58:24 59:2 60:11</p> <p>therapy 15:14 20:14,20 22:3 25:22 31:12,15 33:3 42:24 46:2,20 51:8 53:7 54:9 57:16 58:16,18 59:10 60:1 61:4,5</p> <p>thing 31:4,8,9 49:1 58:16</p> <p>things 9:5,11 22:3 26:24 27:21 28:19 31:20 32:8 36:23 42:10 47:23 49:20 50:14 60:16</p> <p>thinking 19:10 31:13 32:13 60:14</p> <p>thoughts 8:22 26:10 37:1 44:4</p> <p>throw 53:20</p> <p>ticking 30:17</p> <p>time 6:6 12:6 17:23 24:4 25:19 28:5 36:6 44:10 50:10 61:9</p> <p>times 7:24</p> <p>TNF-ALPHA 37:20</p> <p>today 11:18 53:18 61:11</p> <p>tolerated 59:1</p> <p>tomorrow 29:22</p> <p>ton 51:9</p> <p>tons 39:20</p> <p>tools 55:22</p> <p>top 14:14 15:5 27:18</p> <p>totally 10:3</p> <p>toxic 19:22 60:12</p> <p>toxicities 28:13,23 53:4 58:17,18</p> <p>toxicity 20:13,15,16,22 21:16 28:15 30:21 40:7 51:10 52:16 57:21</p> <p>traction 46:3</p> <p>tracts 37:5</p> <p>traditional 19:24 47:5</p>
<hr/> <p style="text-align: center;">T</p> <hr/>		
<p>takes 9:11</p> <p>talk 11:18 26:3 35:21 39:20 58:2</p> <p>talked 30:18 42:2</p> <p>talking 9:3 39:24 40:17 53:7 54:11</p> <p>taper 10:8 23:1</p> <p>tapering 22:23 42:11</p> <p>target 6:19</p> <p>ten 41:17 51:23</p>		

trained 43:13
transaminitis 50:6
transplant 12:3,7,17 16:5 23:24
 24:12 27:4 44:22 51:21 52:3,9,12,20,
 22 53:6,14 54:20 55:6,7,20 56:13,23
 57:4,12,19
transplant-related 55:13
transplantation 10:24
transplanters 52:6 54:5
transplanting 52:14
transplants 11:7 12:12,14,19 13:4,
 10 43:22 52:24 53:20 54:7,16 55:12
 56:8 57:8
treat 15:3 17:2,17 19:9 49:4 57:21
treated 14:22
treating 10:2 14:15 25:7 26:9 27:17
treatment 6:18,20,22 15:8 19:16,20
 26:4 29:15 34:15,20,24 35:3 58:5
treatments 30:7 34:17
tremendously 49:24
trends 6:5 13:21 51:20,21 52:3
 53:14,17,23
trial 7:17 11:19 27:2,8,16,22 30:10
 31:24 32:1,10 34:4 36:3,4 38:18
 39:10,13 41:19 42:5 43:24 44:18,20
 45:4 46:11
trials 6:24 7:5,7 31:20 37:24 47:20
trouble 7:3
truth 31:11
Ts 51:22 52:8,12,19 53:2
turn 51:14
twelve 59:13
twos 27:9
type 30:2
typical 33:8

U

ultimate 30:5
underlying 60:24
understand 45:9
understanding 32:8

undertaken 32:18
unintelligible 19:14 29:13 33:19
unique 10:23 45:22
universal 31:10
unmet 16:16,22 20:3
uphill 46:2 49:15
upside 39:4
Ursodiol 50:14
usage 33:21 52:3 53:17,23

V

vanishingly 47:23
variable 21:9
variety 11:17
vast 54:14 59:22
venetoclax 55:2
versed 55:1
verses 25:18
versus 14:4 21:23 22:12,22 25:20
 30:6 38:18 39:17 47:20
viral 18:22
vulnerable 21:4

W

walk 53:4
wanted 41:2
wary 45:19 46:18
wasting 59:5
ways 32:14 34:2 41:14 50:5 57:15
week 7:24
weeks 28:7
well-conducted 31:19
wind 12:8 23:5
wonderful 54:8
work 6:24 18:10 20:2 29:9 31:21 32:9
 41:20 42:1 43:9 45:20
working 9:7 27:10 28:7 37:5 54:22
works 35:8 47:24 49:7

worried 18:21,24
worrisome 6:19
worse 52:17
worst 37:1,2
wrong 26:2

Y

year 33:20 53:20,21 57:1 59:12
years 26:23 41:17 48:17 51:23 53:8,
 23 57:2 60:2,3
York 56:20
YOUNG 35:19 36:13 38:2,10,13
 40:16,20 51:16

Z

zone 12:4