

UNITED STATES PATENT AND TRADEMARK OFFICE

BEFORE THE PATENT TRIAL AND APPEAL BOARD

APOTEX INC.,
Petitioner,

v.

CELGENE CORPORATION,
Patent Owner

Case IPR2023-00512
Patent 8,846,628

EXPERT DECLARATION OF WILLIAM G. BLUM, MD

CELGENE 2053
APOTEX v. CELGENE
IPR2023-00512

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I, William G. Blum, M.D., declare as follows:

I. INTRODUCTION AND BACKGROUND

1. I have been retained by counsel for Celgene Corporation (“Patent Owner”) as an expert in *Apotex Inc. v. Celgene Corporation*, No. IPR2023-00512, challenging claims 1, 2, 6-9, 11-28, 32-36, and 38-43 of U.S. Patent No. 8,846,628 (“the ’628 patent”).

2. I understand that Apotex Inc. (“Apotex”) have filed an Inter Partes Review (IPR) at the USPTO challenging claims 1, 2, 6-9, 11-28, 32-36, and 38-43 of the ’628 patent.

A. Qualifications and Experience

3. I am a board-certified hematologist-oncologist specializing in treatment of acute myeloid leukemia (AML), myelodysplastic syndromes (MDS), acute lymphoblastic leukemia and other myeloid malignancies. I have over 20 years of experience treating patients in this field including 19 years treating patients with injectable 5-azacytidine (Vidaza®) and 3 years treating patients with oral 5-azacytidine (Onureg®).

4. I am currently a professor in the Department of Hematology and Medical Oncology at Emory University School of Medicine. I serve as Director of the Acute Leukemia Program at Winship Cancer Institute and also serve on the clinical team in the Bone Marrow and Stem Cell Transplant Center at Winship. I

have worked at Emory since 2017. Prior to working at Emory, I was a Professor in the Division of Hematology in the Department of Internal Medicine at The Ohio State University - James Cancer Hospital and Solove Research Institute from 2003-2017.

5. I am a member of several professional organizations including American Society of Hematology, American Association of Cancer Research, American Society of Clinical Oncology, American Society of Bone Marrow Transplantation and Alliance Cooperative Group for Clinical Trials in Oncology. Additionally, I am a cadre member of the Alliance Leukemia Committee and principal investigator/study chair for The Leukemia & Lymphoma Society's Beat AML Master Trial. I have been an invited speaker at international meetings of the American Society of Hematology, American Society of Clinical Oncology, European Hematology Association, and the Tandem Transplant meetings of the Center for International Blood and Marrow Transplant Research/American Society of Blood and Marrow Transplantation, among others.

6. I received my Medical Degree from the Medical School of Georgia in Augusta, Georgia, completed my residency at the University of Virginia in Charlottesville, Virginia, and completed my fellowship in medical oncology at Washington University in St. Louis, Missouri.

7. My curriculum vitae, which lists my professional experience and qualifications in greater detail, is attached hereto as **Appendix A**.

B. Compensation

8. I am being compensated for my time in connection with this matter at my standard consulting rate, which is \$1,000.00 per hour. My compensation is not dependent in any way upon the substance of my opinions or the outcome of this matter.

II. PERSON OF ORDINARY SKILL IN THE ART

9. I understand that a POSA relating to the subject matter of the '628 patent would have had (1) a Pharm.D., and/or a Ph.D. in pharmaceutical sciences, biomolecular engineering, chemical engineering, chemistry, or related discipline or an M.D.; and (2) at least two years of experience with oncology, medicine, pharmaceutical design, or formulation of oral dosage forms. As of December 2008 I would have been a POSA.¹

¹ I understand Petitioner's experts, Drs. Buckton and Batchelor, each put forth identical definitions of a POSA, and that they both opine that a POSA as of December 5, 2008 would have had (1) a Pharm.D., or a Ph.D. in pharmaceutical sciences, chemical engineering, chemistry, or related discipline; and (2) at least two to four years of experience with pharmaceutical design, formulation,

III. BASIS OF OPINIONS

10. In forming my opinions set forth in this Declaration, I have considered and relied on my education and experience in the fields of oncology and treating AML and MDS patients. I have also relied on the materials listed in **Appendix B**.

IV. BACKGROUND

A. AML and MDS

11. In the early 2000s, doctors understood that “[t]he prognosis for patients with myelodysplastic syndrome (MDS) is grim[.]” Ex.2092(Kornblith), 2441. Every year, more than 10,000 patients in the United States are diagnosed with MDS (Ex.2098(Ma), Abstract), which is a collection of blood cell disorders caused by malfunctioning bone marrow (Ex.2064(Altman), 197). Most MDS patients die from bleeding or infection sometimes with progression of MDS into acute myeloid leukemia (AML or acute myelogenous leukemia).

development, and/or manufacturing of oral dosage forms. I further understand that Dr. Buckton states a POSA would have consulted with a team including at least a clinician. I am (and was since before the priority dates of the '628 patent) a clinician that would have been consulted. My opinions would not change under this definition.

Ex.2018(Kaminskas), 181. For the highest risk patients, median survival rate is between only six and twelve months. Ex.2092(Kornblith), 2441.

12. In adults, AML is the most common type of acute leukemia with about 20,000 patients diagnosed in the United States each year. Ex.2072(Choi); Ex.2071(Carter), 1. It is an aggressive form of cancer that “has the lowest survival rate of all leukemia types...” Ex.2072(Choi), 1416; Ex.2142(European Medicines Report), 8. In children, it is the second most common acute leukemia. Ex.2064(Altman), 16; Ex.2071(Carter), 1. AML results in the growth of abnormal immature blood cells in the bone marrow. Ex.2064(Altman), 16-17. AML’s poor prognosis is stark: three-quarters of AML patients die within five years, and among older patients long-term survival is uncommon. Ex.2073(Cogle), 1405.

13. As of December 2008, standard therapy for AML was intensive “induction” therapy designed to achieve remission followed by short-term intensive “consolidation” therapy to try to eradicate undetectable residual leukemia. Ex.2071(Carter), 2-3; Ex. 2111(Reville), 1. Risk of relapse, though, remained high, occurring in more than 50% of adults. Ex. 2111(Reville), 1. At that time, the FDA had only approved one oral therapy (lenalidomide) to treat MDS. Ex.2076(Curtiss), 573; Ex.2071(Carter). However, although low intensity, prolonged so-called maintenance therapy was established in acute lymphoblastic leukemia, there was no approved oral or maintenance therapies for AML.

Ex.2076(Curtiss), 573; Ex.2071(Carter). Many different trials, including CALGB 10503 of which I was the national study chair, tried to establish efficacy of various maintenance therapies with chemotherapy but were all ineffective. *See e.g.*, Ex.2136(Blum), Abstract.

B. 5-Azacytidine Treatments

14. In 2004, the FDA first approved 5-azacytidine as the injectable drug, Vidaza®, to treat MDS. *See* Ex.1007(Vidaza Label 2004); Ex.2018(Kaminskas), 176. Clinicians were hopeful that further development would result in “non-toxic therapies with convenient dose schedules, such as oral administration, [to] provide new tools for cancer prevention and maintenance.” Ex.2087(Grønbæk), 395.

15. In 2007, the FDA granted Pharmion Fast Track designation for oral 5-azacytidine. *See* Ex.1011-0005(Pharmion-PR II). The Fast Track program seeks “to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions” and have “the potential to address unmet medical needs.” Ex.1011-0005(Pharmion-PR II). Over a decade and a half after approving the injectable formulation of 5-azacytidine (Vidaza®), the FDA finally approved the first oral formulation of 5-azacytidine as Onureg® in September 2020. *See* Ex.2055 (FDA Approves Onureg®); Ex.1007(Vidaza® Label 2004); Ex.2062(Onureg® Label).

16. Onureg® is an immediate release (*i.e.*, non-enterically coated) (*see* Ex.2142(European Medicines Report, 15) tablet and is also the first FDA approved drug for maintenance treatment of patients with AML who achieved remission following intensive induction chemotherapy and are not able to complete intensive curative therapy such as allogeneic donor cell transplant. (Ex.2062(Onureg® Label), 1). Canada and Europe quickly followed suit, approving Onureg® in January and June 2021, respectively. Ex.2056(Canadian Product 200mg Onureg® Product Information); Ex.2057(Canadian Product 300mg Onureg® Product Information); Ex.2058(Canadian Product Monograph Onureg®); Ex.2059(European Commission Decision 17.6.21). The FDA also granted Onureg® Orphan Drug designation, which was set up to provide incentives for the research and development of drugs to treat uncommon diseases. *See* Ex.2140(Finkel), 313.

C. The '628 Patent

17. The '628 patent is directed to “pharmaceutical compositions comprising cytidine analogs,” specifically 5-azacytidine, “for oral administration, wherein the compositions release the cytidine analog...substantially in the stomach” as well as “methods of treating diseases and disorders using the oral formulations provided herein.” Ex.1001('628 patent), Abstract. The patent includes two independent claims: claim 1 and claim 28.

18. Independent claim 1 of the '628 patent is reproduced below:

1. A pharmaceutical composition for oral administration comprising a therapeutically effective amount of 5-azacytidine and at least one pharmaceutically acceptable excipient, wherein the composition is a non-enteric coated tablet.

19. Independent claim 28 of the '628 patent is reproduced below:

28. A method for treating one or more symptoms of a disease associated with abnormal cell proliferation, comprising orally administering to a subject in need thereof a pharmaceutical composition comprising a therapeutically effective amount of 5-azacytidine and at least one pharmaceutically acceptable excipient, wherein the composition is a non-enteric coated tablet, and wherein the disease associated with abnormal cell proliferation is myelodysplastic syndrome or acute myelogenous leukemia.

V. ONUREG®

20. I understand that FDA-approved Onureg® is a pharmaceutical composition for oral administration comprising a therapeutically effective amount of 5-azacytidine and at least one pharmaceutically acceptable excipient, wherein the composition is a non-enteric coated tablet. *See, e.g.,* Ex.2062(Onureg® Label). Additionally, I understand that the FDA-approved Onureg® for “continued treatment of adult patients with acute myeloid leukemia who achieved first complete remission (CR) or complete remission with incomplete blood count recovery (CRi) following intensive induction chemotherapy and are not able to complete intensive curative therapy.” Ex.2062(Onureg® Label).

VI. ONUREG® EMBODIES THE '628 PATENT'S CLAIMS

21. I have spoken to Cory Berkland, Ph.D., who I understand analyzed the literature about Onureg® and the claims, and from this conversation I understand Onureg® embodies the claims of the '628 patent.

VII. A CLINICIAN WOULD NOT HAVE LOOKED TO PETITIONER'S REFERENCES FOR TREATMENT OPTIONS IN DECEMBER 2008

22. I understand that Apotex relies on the following references:

- Ionescu (Ex.1004)
- Atadja (Ex.1005)
- Gibson (Ex.1006)
- Pharmion-PR (Ex.1010).

23. I have reviewed these references and provide the following comments from the perspective of an oncologist treating MDS and/or AML patients with 5-azacytidine during the relevant time period.

A. Clinicians Would Not Have Pursued An Unproven Therapeutic Formulation

24. The route of administering a pharmaceutical compound influences its biological impact. “Bioavailability is highly dependent on both the route of administration and the drug formulation,” and factors including the absorption process and “metabolism [of the drug] prior to reaching the systemic circulation” may reduce a drug’s bioavailability. Ex.2075(Craig), 50. Indeed, by December

2008 an oncologist would have been aware of many injectable chemotherapeutics that were not approved for oral administration, several of which belong to the same class of nucleotide analogs as 5-azacytidine. *See, e.g.*, Ex.2137(Cytarabine), 8 (disclosing an injectable but not oral cytidine analog approved to treat acute nonlymphocytic leukemia that is acute myeloid leukemia); Ex.2138(Dacogen), 1, 6 (disclosing a different injectable but not oral cytidine analog approved to treat MDS); Ex.2139(Mitoxantrone), 2, 14 (disclosing a non-nucleoside injectable but not oral formulation approved to treat acute nonlymphocytic leukemia). A clinician would have understood that the therapeutic effectiveness of a drug can depend on its administration route and is highly unpredictable when administered in a manner inconsistent with the established clinical data. Without more information, therapeutic effectiveness of a drug given intravenously or subcutaneously does not indicate that the same drug would be therapeutically effective if administered orally.

25. None of Ionescu, Atadja, Gibson, and Pharmion-PR would have provided any reason for an oncologist to expect that an oral formulation of 5-azacytidine would have been tolerable and therapeutically effective for a patient.

1. Ionescu (Ex.1004)

26. Ionescu “relates to the isolation of crystalline polymorphic and pseudopolymorphic forms of 5-azacytidine....” Ex.1004-0003(Ionescu), 1:8-10. It

notes that “5-azacytidine may be used in the treatment of disease, including the treatment of myelodysplastic syndromes” (Ex.1004-0003(Ionescu), 1:10-11), but only references working examples of injectable formulations of 5-azacytidine suspended in a water-based solution, *i.e.*, Vidaza®. *See, e.g.*, Ex.1004-0011.

Otherwise, Ionescu provides only a general disclosure of other aspects of 5-azacytidine pharmaceutical compositions such as possible excipients (Ex.1004(Ionescu), 2:26-28), generic and hypothetical formulations (Ex.1004(Ionescu), 12:11-14:29), and broad ranges of potential dosages (Ex.1004(Ionescu), 14:22-25). Nowhere does Ionescu disclose a working oral formulation of 5-azacytidine (such as Onureg®); a safe, effective, tolerable, and non-hazardous dosage; or any data showing efficacy upon oral administration. Thus, a clinician would not have been motivated by Ionescu to treat a patient with AML or MDS with oral 5-azacytidine, nor would they have had any expectation of success using such a treatment.

2. Atadja (Ex.1005)

27. Atadja discloses over 130 various chemotherapeutics to be combined with histone deacetylase inhibitors. Ex.1005-0002-0028(Atadja). Atadja provides a blanket statement that any of “the compounds or the pharmaceutically acceptable salts thereof, are administered as an oral pharmaceutical formulation in the form of a tablet, capsule or syrup; or *as parenteral injections if appropriate.*” Ex.1005-

0028(Atadja) (emphasis added). An oncologist confronted with the over 130 chemotherapeutics disclosed in Atadja would have understood that each chemotherapeutic agent would be administered based on the clinical data and FDA approvals for each respective drug at the time. For example, an oncologist would have understood the FDA had approved certain agents listed in Atadja as tablets. *See, e.g.,* Ex.1005-0004-0005(Atadja); Ex.2133(Exemestane); Ex.2134(Anastrozole), 1; Ex.2135(Letrozole), 1. On the other hand, an oncologist in December 2008 would have recognized that the disclosure of “parenteral injections [would have been] appropriate” for 5-azacytidine since ***the only FDA-approved formulation of 5-azacytidine was injectable Vidaza®***. Ex.1005-0028(Atadja); Ex.1007(Vidaza® Label 2004); Ex.1008(Vidaza® Label 2007). An oncologist would have understood Atadja’s blanket disclosure of formulations in the specific context of the existing data for each of the dozens of agents listed and applied that understanding to determine which formulation was appropriate for which agent.

3. Gibson (Ex.1006)

28. Gibson is a general formulation textbook without mention of 5-azacytidine, MDS, or AML. Gibson would not have provided a clinician with information on how to administer 5-azacytidine in a therapeutically effective amount to treat a patient.

4. Pharmion-PR (Ex.1010)

29. An oncologist would not have understood Pharmion-PR to disclose a therapeutically effective oral formulation of 5-azacytidine, such as Onureg®. Pharmion-PR merely reports “preliminary results” of a pilot study “designed to assess the safety, tolerability, and pharmacokinetics” of administration of an undescribed oral formulation of 5-azacytidine. Ex.1010-0005, -0006(Pharmion-PR). An oncologist would have understood that these results do not indicate that the formulation is therapeutically effective but merely that it was “well-tolerated and quantifiable in plasma.” *See* Ex.1010-0005(Pharmion-PR). Indeed, the preliminary study concludes that the undescribed formulation results in only 18% bioavailability relative to that of subcutaneously administered Vidaza® (Ex.1010-0005(Pharmion-PR)), and the reference provides no evidence that the administered dose was therapeutically effective. In fact, a clinician would have understood that “the relationship between drug intake and a clinical response is highly complex,” and “deviations between drug response within or between individuals may be ascribed either to product bioavailability..., drug pharmacokinetics..., or the particular concentration-effect relationship.” Ex2141(Martinez), 620. A clinician would have understood that bioavailability is not necessarily an indication of therapeutic effectiveness as other factors may affect clinical responses. For example, a clinician would have looked to dose escalation studies that evaluate an

efficacious dose to ascertain whether the dose also has an acceptable toxicity profile at that dose.

30. Additionally, a clinician would have recognized that Pharmion-PR's "preliminary results" "involve a number of known and unknown risks and uncertainties that could cause the final results to differ significantly from the results summarized...." Ex.1010-0006(Pharmion-PR). A clinician would have understood and agreed that Pharmion-PR's "*preliminary results may not be confirmed upon the full analysis* of the detailed results of a trial and additional information relating to the safety, *efficacy* or tolerability of oral azacitidine[.]" Ex.1010-0006(Pharmion-PR) (emphasis added).

31. An oncologist in December 2008 would not have pursued an oral non-enteric coated tablet of 5-azacytidine as a therapeutic for a subject with AML or MDS based on the prior art at least because at that time there was no data to establish that an oral tablet of the drug could be given at an efficacious dose in a safe manner.

VIII. AS OF DECEMBER 2008 THERE WAS A LONG-FELT BUT UNMET NEED FOR ONUREG®

A. There Was an Unmet Need for A Therapeutically Effective Oral Formulation of 5-Azacytidine

32. As of 2008, clinicians were desirous of "more specific and non-toxic therapies with convenient dose schedules, such as oral administration, [to] provide

new tools for cancer prevention and maintenance.” Ex.2087(Grønbaek), 395.

Clinicians and researchers understood that 5-azacytidine was potentially useful in treating cancers as soon as it was first synthesized. *See, e.g.*, Ex.2012(Čihák), 2100. However, for many decades, clinicians believed the development of an oral formulation was fraught with difficulty. *See* Ex.2029(Stresemann), 8 (disclosing difficulty in formulating 5-azacytidine “interferes with [its] potential oral administration”); *see also* Ex.1014(Chan), 807 (disclosing 5-azacytidine’s “proper formulation [was] a problem in its clinical use”); Ex.2003(Aparicio), 631 (disclosing its “significant restrictions on dosing schedule adjustments” inhibited the creation of a “dramatically successful” formulation of 5-azacytidine). The FDA approved Vidaza®, an injectable formulation of 5-azacytidine, in 2004 (Ex.1007(Vidaza Label 2004)), but injection protocols dictated by 5-azacytidine’s instability had been associated with “severe gastrointestinal toxicity....” (Ex.2004(Beisler), 281). *See also* Ex.2125(Vigil), 227 (injectable 5-azacytidine caused discomfort and other injection-site reactions in patients); Ex.2118(Sorrentino), 20.

33. Thus, for over forty years clinicians wanted for a therapeutically effective, oral formulation of 5-azacytidine that was only realized by Onureg®.

B. There Was a Long-Felt Unmet Need for a Continued Therapy for AML

34. Since at least 1968, clinicians have recognized that “[m]ost patients with AML... will relapse....” Ex.2073(Cogle), 1409; Ex.2080(Ellison), 509; *see also* Ex.2110(Récher), 828-829 (“Preventing relapse after induction and consolidation therapy in AML has long been a major challenge[.]”). The outcomes for recovering AML patients that experience early relapse “are dismal.” Ex.2125(Vigil), 224); *see also* Ex.2082(Frisch), 683. Clinicians understood that “[t]o improve outcomes, there is a need to prolong remissions” through maintenance therapies. Ex.2073(Cogle), 1409; *see also* Ex.2118(Sorrentino), 20 (“An oral formulation [of 5-azacytidine] is clinically desired due to the opportunity of delivering at lower systemic doses over a more prolonged schedule.”). At the time of the invention of the claims at issue here, “there was an unmet need for a therapy to maintain remission in such patients” because “a convenient, safe and effective option with proven capacity to improve survival was not available.” Ex.2119(Southall), 1.

35. The search for an effective therapy for AML patients in remission had been ongoing for about 40 years before the invention of the ’628 patent and nearly 50 years before the FDA approved Onureg® in 2020. *See, e.g.*, Ex.2080(Ellisson) (studying the effects of subcutaneous arabinosyl cytosine maintenance treatments

in AML patients after remission); *see also* Ex.2108(Preisler), 1442-1443 (assessing subcutaneous cytosine arabinoside in rotating combination with 6-thioguanine, prednisone and vincristine, and daunorubicin in AML patients after remission); Ex.2097(Löwenberg) (evaluating effects of cytarabine as a maintenance therapy in AML patients after remission).

36. Before the invention of the claims at issue and the launch of Onureg®, clinicians had not made substantial progress in identifying a maintenance therapy for AML. “For decades, although several trials with different strategies of maintenance therapy have been conducted to reduce the relapse of disease in patients, these trials have failed to show the improvement of outcomes. Consequentially, maintenance chemotherapy is not a standard therapy for AML except for acute promyelocytic leukemia (APL).” Ex.2072(Choi), 1416; *see also* Ex.2073(Cogle), 1410 (noting in 2015 that “there is an unmet need for additional low-intensity treatment options for patients with MDS or AML....”). Clinicians recognized a need for a therapeutic agent approved for “continuing therapy beyond the time of first response....” Ex.2093(Lammers), 1196.

37. With the launch of Onureg®, such a long-desired continuous therapy was realized for the first time. Onureg® fulfilled this previously unmet need, “bec[oming] the first FDA approved drug specifically as maintenance treatment for AML patients....” Ex.2095(Liu), 13. With its approval, clinicians “[a]t last, [had]

an effective treatment that can be given in the post-remission setting to help keep AML patients in remission and improve their survival.” Ex.2130(Weill Cornell), 2.

38. Thus, a multidecade search for a continued therapy for AML finally came to fruition with Onureg®.

C. The FDA Designated Oral 5-Azacytidine for Fast Track Status

39. In 2007, the FDA granted to Pharmion Fast Track designation for oral 5-azacytidine. *See* Ex.1011-0005(Pharmion-PR II). Fast Track designation is “designed to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs.” Ex.1011-0005(Pharmion-PR II). This designation provided “[t]he opportunity to explore the biological and clinical consequences of continuous oral dosing of Azacitidine” for the first time. Ex.1011-0005(Pharmion-PR II); *see also id.* at -0006 (“An oral dosage formulation of Azacitidine... would enable the evaluation of a low-dose regimen... [and] long-term or maintenance therapy.”). With this designation the FDA formally recognized what clinicians had previously understood—an unmet need for oral 5-azacytidine treatments. Section VIII.A.

40. It took over four decades to develop Onureg®, the much-desired oral formulation of 5-azacytidine. These extended efforts reflected the desires of

clinicians, researchers, and the U.S. government to provide a therapeutically effective oral formulation for the continued treatment of AML patients in remission. Only once the inventors developed the claimed invention, which was launched as Onureg®, was this long-felt need for a maintenance therapy for AML met. Section VIII.B.

IX. THE MEDICAL INDUSTRY PRAISED ONUREG®

41. Clinicians recognized Onureg® as a major development in the field of oncology even before its FDA-approval. Ex.2104(Perl), 2287; Ex.2068(Bankhead), 2. They praised oral 5-azacytidine, approved as Onureg®, as a “transformative” and “very significant advance” that “represent[s] a potential new standard of care for patients” and would “change practice once [it became] available.” Ex.2103(Oral CC-486), 2; Ex.2114(Slater), 2. Clinical results using the non-enteric coated tablets of 5-azacytidine provided clinicians with a tool to “improve[] relapse-free survival and overall survival” in older AML patients for the “first time.” Ex.2103(Oral CC-486), 4. This led clinicians to hail Pharmion’s non-enteric coated oral 5-azacytidine tablets, which would be approved as Onureg®, as “practice-changing” (Ex.2103(Oral CC-486), 4) and recommending doctors “strongly consider” treating patients with Onureg® (Ex.2129(Wei), E86). In fact, clinicians recognized Onureg®’s approval as ushering in a “period of breakthrough drug approvals for AML....” Ex.2094(Lin 2022), 1; *see also*

Ex.2089(Kantarjian), 581 (“We are witnessing an ongoing ‘slow-motion’ revolution in AML research and therapy, with the approval of 9 agents for different AML indications since 2017” including “the oral [hypomethylating agent] azacitidine[.]”).

42. As noted above, these breakthroughs extended to providing continued therapy to patients. Section IV.B. “Historically, maintenance therapy with various agents consistently failed to demonstrate [an overall survival] advantage.”

Ex.2082(Frisch), 683; *see also* Ex.2150(Huls), 1457, 1458 (noting that subcutaneous 5-azacytidine administered as a maintenance therapy did not improve overall survival in patients). Indeed, clinicians long recognized that the major issue for the treatment of AML was the lack of effective maintenance therapies.

See Ex.2094(Lin 2022), 13; *see also*, Ex.2110(Récher), 828-829. Onureg® changed this, “bec[oming] the first FDA approved drug specifically as maintenance treatment for AML patients....” Ex.2095(Liu), 13. Clinicians noted its success as “[t]he biggest advance in AML maintenance currently....”

Ex.2111(Reville), 1. Further, clinicians recognized Onureg®’s approval as potentially paradigm-changing. *See* Ex.2111(Reville), 7 (stating that the post-complete remission care “paradigm maybe [*sic*] set to change” thanks to the approval of Onureg®); Ex.2081-0001(FDA Approves Oral Azacitidine) (clinician stating that the approval of Onureg® “should help establish continued treatment

with azacitidine as a standard component of AML therapy for adults who achieved first complete remission” but cannot proceed to intensive curative therapy or transplantation).

43. The success of Onureg® highlights the potential for routes of administration that facilitate new protocols including low, continuous dosing of chemotherapeutics, such as 5-azacytidine, that permit “a more sustained cellular exposure” yet are well tolerated. Ex.2102(Odenike), 461; *see also*, Ex.2084(Garcia-Manero 2011), 2526 (the lower comparable bioavailability of oral 5-azacytidine results in similar rates of epigenetic modification associated with subcutaneous administration); Ex.2073(Cogle), 1404 (These “extended dosing schedules enabled by oral administration” overcame 5-azacytidine’s instability and limited bioavailability “by increasing exposure to cycling malignant cells” and thereby “potential[ly] ... enhance[ing] clinical activity of azacitidine.”). Onureg® has succeeded by facilitating previously unavailable extended treatment regimens and maintenance therapies for patients. Ex.2073(Cogle) at 1406 (“An oral formulation [of 5-azacytidine] improves convenience and eliminates injection-site reactions but also enables testing of novel, longer term, low-dose schedules that may enhance therapeutic activity of azacitidine by increasing exposure to cycling malignant cells.”).

44. Clinicians have also praised Onureg®’s removal of physical and logistical challenges for patients. “One of the key challenges in treating acute myeloid leukemia is to prevent relapse after remission has been achieved,” which “means that developing an effective maintenance treatment is very important.” Ex.2091(Kipp), Abstract. Because such “[m]aintenance treatment is given for a prolonged period,” “it needs to be easy to give and well tolerated.” Ex.2091(Kipp), Abstract; *see also* Ex.2086(Griffiths), Abstract (“For patients who respond to treatment, ongoing monthly maintenance visits represent a considerable burden to quality of life, and for those who are early in therapy, these sequential visits may tax transportation and financial resources[.]”). Understanding these burdens, clinicians have praised Onureg® as an “important therapy” and “the first drug that has been shown to improve survival as maintenance therapy for acute myeloid leukemia patients.” Ex.2091(Kipp) Abstract; *see also* Ex.2086(Griffiths), Abstract (“The availability of oral [forms of a hypomethylating agents, such as 5-azacytidine] may support the optimal application of these agents by contributing to adherence and lessening the burden of therapy....”); Ex.2115(Sochacka-Ćwikła), 15 (praising Onureg®’s oral formulation as “facilitate[ing] the drug administration, reduc[ing] the potential side effects associated with subcutaneous injection, and allow[ing] for the expansion of treatment regimens by searching for alternative doses or combinations of therapies.”); Ex.2120(Steensma), 388 (“Oral azacitidine

might offer some advantages for those patients” “who must travel from a distance to a clinical facility to receive [5-azacytidine] injections multiple consecutive days each month.”); *see also* Ex.2076(Curtiss), 570, 573, 575 (patients must travel to a doctors for seven days of Vidaza® injections every four weeks); Ex.2118(Sorretino).

45. This praise extends to Onureg®’s ability to reduce side effects. Injectable formulations are associated with patient discomfort and disadvantageous injection-site reactions. *See* Ex.1001(’628 patent), 3:50-54; Ex.2125(Vigil), 227; Ex.2118(Sorrentino), 20. The availability of oral Onureg® allows for self-administration that “reduc[es] the potential side effects associated with subcutaneous injection....” Ex.2115(Sochacka-Ćwikła), 15; *see also* Ex.2118(Sorrentino), 20 (“the route of oral administration will alleviate the injection site reactions associated with [V]idaza.”). As a result, clinicians have praised Onureg® as potentially “enhanc[ing] patient convenience compared with an injectable formulation.” Ex.2073(Cogle), 1406.

46. Thus, Onureg® provides the first maintenance therapy for AML patients ever, enabling prolonged low-dosage administration, and providing the least burdensome form of a maintenance therapy benefiting patients and improving their survival.

47. I have also been using Onureg® for 3 years in my own oncology practice. As of October 2008, I have prescribed or administered Onureg® as a continued therapy to around 20 AML patients including my oversight of other physicians in my role as a leader in our program. Before Onureg®, no options for AML patients existed after completing intensive initial induction and consolidation chemotherapy other than bone marrow/stem cell transplantation, despite the high level of relapse. *See, e.g.*, Ex.2073(Cogle), 1409; Ex.2125(Vigil), 224; Ex.2110(Récher), 828-829; Ex.2082(Frisch), 683. Notably, patients enrolled in the pivotal Onureg® trial were deemed not to be candidates for transplantation by their treating physicians. My patients have benefited from the availability of Onureg® for maintenance therapy.

X. AVAILABILITY FOR CROSS-EXAMINATION

48. In signing this declaration, I recognize that the declaration will be filed as evidence in a contested case before the Patent Trial and Appeal Board of the United States Patent and Trademark Office. I also recognize that I may be subject to cross examination in the case and that cross examination will take place within the United States. If cross examination is required of me, I will appear for cross examination within the United States during the time allotted for cross examination.

XI. RIGHT TO SUPPLEMENT

49. I reserve the right to supplement my opinions in the future to respond to any arguments that Petitioner raises and to take into account new information as it becomes available to me.

XII. JURAT

50. I declare that all statements made herein of my own knowledge are true and that all statements made on information and belief are believed to be true; and further that these statements were made with the knowledge that willful false statements and the like so made are punishable by fine or imprisonment, or both, under Section 1001 of Title 18 of the United States Code.

Signed: _____

Dr. William G. Blum

Date: _____

10/12/23

APPENDIX A

EMORY UNIVERSITY SCHOOL OF MEDICINE

Revised: April 11, 2023

1. Name: William G. Blum, M.D.
2. Office Address: 1365 Clifton Rd, Room C1150, Atlanta GA, 30322
Telephone: 404-778-3932
Fax: 404-778-4755
3. E-mail Address: wblum@emory.edu
4. Citizenship: USA
5. Current Titles and Affiliations:
 - a. Academic Appointments:
 - i. Primary Appointments: Professor, Department of Hematology and Oncology, Emory University School of Medicine, 9/2017
 - b. Clinical Appointments:
Director, Acute Leukemia Program, Winship Cancer Institute of Emory University, 9/2017
 - c. Other Administrative Appointments:
Director, Cellular Therapy Working Group, Winship Cancer Institute 9/2017-10/2021
Director, Leukemia Working Group, Winship Cancer Institute 9/2017-present
6. Previous Academic and Professional Appointments:
Assistant Professor of Medicine, Division of Hematology/ Oncology, James Cancer Hospital, The Ohio State University (OSU); Columbus, OH, 2003- 2010

Cadre Member, Alliance for Clinical Trials in Oncology Leukemia Committee, 2008-present

Chair – OSU Comprehensive Cancer Center (CCC) Data and Safety Monitoring Committee (DSMC), 2010-2017

Associate Professor of Medicine with Tenure, Division of Hematology, James Cancer Hospital, The Ohio State University, Columbus, OH, 2010-2015

Section Head, Acute Myeloid Leukemia and Myelodysplastic Syndromes (all Division sections dissolved 2015), Division of Hematology, The Ohio State University School of Medicine; Columbus, OH, 2013-2015

Professor of Medicine with Tenure, Division of Hematology, James Cancer Hospital, The Ohio State University, Columbus, OH, 2015-2017
7. Licensures/Boards:
Medical License, Ohio, 2003-present
Medical License, Georgia, 2017-present
8. Specialty Boards:
Certification in Internal Medicine, 2001
Certification in Hematology, 2004, 2014

Certification in Medical Oncology, 2004

9. Education:
1993, B.S., University of Notre Dame
1997, M.D., Medical College of Georgia
10. Postgraduate Training:
1997-2000, Internal Medicine Internship and Residency, University of Virginia, Charlottesville, VA
2000-2003, Fellowship in Hematology and Oncology, Washington University in St. Louis, St. Louis, MO
11. Committee Memberships:
 - a. National and International:

Cadre Member, Alliance for Clinical Trials in Oncology (Alliance, formerly CALGB) Leukemia, Committee, 2008-present

Ad hoc member, Audit Committee, Cancer and Leukemia Group B, 2006–2012

Review panel member, National Comprehensive Cancer Network (NCCN), development of first national clinical practice guidelines for acute lymphoblastic leukemia, 2012-2015

Review panel member, National Comprehensive Cancer Network (NCCN), development of national clinical practice guidelines for acute myeloid leukemia, 2016-2017

NCI Pediatric Leukemia and Lymphoma Steering Committee (PLLSC), adult medical oncology member (I resigned after move to new institution), 2017-2018
 - b. Institutional:

Member - Clinical Scientific Review Committee, OSU Comprehensive Cancer Center, 2003-2010, (Vice Chair, 2006-2010)

Clinical Improvement Committee, 2005

Practice Management Committee, 2005 –2006

Member – Hematology/Oncology Fellowship Selection Committee, 2006

Leader – Ohio State Acute Leukemia Working Group, 2009-2017

Chair – OSUCCC Data and Safety Monitoring Committee (DSMC), 2010-2017

Fellowship Competency Committee, 2011

Licensed Healthcare Professionals Credentials Subcommittee, 2011-2013

Member – Ad Hoc Task Force for the Division of Clinical Trials within the Department of Pharmacology, OSU Medical Center, invited by Dean, 2012

FCAP, Emory School of Medicine, 8/2018—8/2021

Winship Invest\$ Executive Review Committee, 2019, 2020

12. Peer Review Activities:

a. Grants:

i. National and International:

NIH Challenge Grants, NIH, 2009
European Organization for Research and Treatment of Cancer (EORTC), 2013
Leukemia Research Programme (UK) Grant, 2015

ii. Regional:

State of North Carolina Biotechnology Grant, 2012, 2013

iii. Institutional:

American Cancer Society Seed Grant (OSU only), 2006-2012, 2016
OSUCCC IRP Clinical/Translational Grant (Pelotonia grant reviewer), 2012-2017
Winship Invest\$ Executive Review Committee, 2019, 2020

b. Manuscripts:

British Journal of Hematology
Annals of Hematology
Blood
Blood Advances
Leukemia
Cancer
Cancer Chemotherapy and Pharmacotherapy
Haematologica
Scandinavian Journal of Infectious Disease
American Journal of Hematology
Leukemia and Lymphoma
Leukemia Research
New England Journal of Medicine
Bone Marrow Transplantation
The Lancet Hematology
JCO Oncology Practice

c. Conference Abstracts:

i. National and International:

Abstract Reviewer, American Society of Hematology Annual Meeting, 2006, 2010, 2012
Abstract Reviewer, American Society of Clinical Oncology Annual Meeting, 2010

ii. Regional/Institutional

Poster Judge, OSU James Annual Scientific Meeting, 2011-2012, 2016

Abstract Judge for OSU James Annual Scientific Meeting, 2011-2013, 2016

Poster Judge for Winship Cancer Center Annual Meeting, 2018

13. Consultantships/Advisory Boards:

Oncology Drug Advisory Committee to the US FDA (ODAC); Consideration of Dacogen for acute myeloid leukemia. External Advisor to Eisai, Inc. FDA White Oak Campus, Silver Spring, MD, 2012

Advisory/consultative activities for industry: MGI Pharma, Pfizer, Astellas, Celator, Johnson and Johnson, Eisai, Abbvie/Genentech-Roche, Boehringer-Ingelheim, Novartis, many others.

Data safety monitoring board (DSMB) for national trials for Celgene, Celator

Response review committee for national trial for Tolero.

Member, National Steering Committee, Abbvie, for use of venetoclax in AML/MDS. 2019-present

14. Honors and Awards:

Summer Research Student Award, lab of Douglas Boyd, The MD Anderson Cancer Center, 1991

Summer Research Student Award, lab of David Harrison, Jackson Laboratory; Bar Harbor, ME, 1992

School of Medicine Scholarship, Medical College of Georgia, 1994-1995

CD Whitaker Scholarship, Medical College of Georgia, 1995

Academic Commendation letter, Medical College of Georgia, 1995

Award for Excellence in Scholarship, Medical College of Georgia, 1995

Alpha Omega Alpha, Medical College of Georgia, 1997

Awarded Jefferson Cup for excellence in resident research, University of Virginia, 2000

Selected Participant, AACR/ASCO Workshop "Methods of Clinical Cancer Research", 2003

Selected Participant, ASH Clinical Research Training Institute (CRTI), 2004

Service Award in the College of Medicine Education Programs, The Ohio State University, 2003-2009

Invited speaker, Education session for myelodysplastic syndromes, American Society of Hematology Annual Meeting, 2010

Invited speaker, American Society of Hematology Annual Meeting "Highlights" lecture series, 2011

Invited consultant, American Society of Hematology "Consult a Colleague" Program, 2013-present

Invited speaker, Education session for AML, American Society of Clinical Oncology Annual Meeting, 2014

Invited speaker, Meet the Professor ticketed session, Tandem Transplant Meetings, February 16, 2023, "Novel treatments in AML, who and when to transplant".

15. Society Memberships:

Alpha Omega Alpha, 1997

American Society of Hematology

American Society of Clinical Oncology

16. Organization of Conferences:

a. National and International:

i. Sessions as Chair:

Chair, Education session for myelodysplastic syndromes, American Society of Hematology Annual Meeting, 2010

17. Community Outreach:

a. General:

Primary care outreach to local indigenous population, outside Ambato, Ecuador (2 weeks), 1995

Medical student sub-internship, 3 months, PCEA Hospital Tumutumu, Karatina, Kenya, 1997

Spring Relay for Life Kick off Rally; YMCA – Grove City, Ohio. Featured Speaker for fundraising rally of team leaders, 2003

Relay for Life; American Cancer Society – Pickerington, Ohio. Featured speaker for fundraising event. Audience 200-300 people and broadcast on live radio, 2003

"Myelodysplastic Syndromes: An Update from ASH" Leukemia & Lymphoma Society, CCONS. Columbus, OH, 2008

Invited Speaker, American Red Cross Platelet Event: How platelets are used to help patients. Columbus Museum of Art, 2015

Keynote speaker, Leukemia and Lymphoma Society Fundraiser, Zack Leukemia Fund, Columbus OH, 2016

Invited Speaker, American Red Cross Platelet Event: Why we give platelets, and thank you!, Columbus Museum of Art, 2016

Keynote speaker, "Rock the Night," James Cancer Hospital and Nationwide Children's Hospital Patient Leukemia Fund fundraiser, Columbus OH, 2017

Invited Speaker, Leukemia and Lymphoma Society Symposium on Hematologic Malignancies, Atlanta GA, 2019 and 2020

Invited Speaker, Update on Beat AML trial, Board of Directors of Georgia Chapter of Leukemia and Lymphoma Society, 2019

“Why the Krebs cycle matters”, lecture to local high school Advanced Biology class, May 2019

New Drugs in AML, CURE regional meeting, 2020

“Leukemias”, annual meeting of the Leukemia and Lymphoma Society (Georgia Chapter), 2018, 2019, 2020

b. Other Media Appearances:

Television interview regarding novel therapeutics in leukemia, Columbus, OH, 2005

NPR Radio Interview (St. Louis Public Radio/90.7 KWMU), University of Missouri, St. Louis. Immune based therapies/trends in AML care and research, 2015

18. Formal Teaching:

a. Medical Student Teaching:

Preceptorship III, Department of Internal Medicine, 2003

Inpatient and Ambulatory Teaching for Medical students, 2003

Lecturer – M1, “Myeloproliferative Disorders”, 2003

Lecturer – M1, “Myeloproliferative Disorders”, 2004

Preceptorship I, Division of Hematology and Oncology, 2004

Preceptorship III Physician Development, Division of Hem/ Oncology, 2004

Lecturer – M1, “Acute Leukemia”, 2005

Preceptorship (Hospital Based), Department of Internal Medicine, 2005

Preceptorship III, College of Medicine, 2005

Preceptorship (Physical Exam), Department of Internal Medicine, 2006

Lecturer - “Acute Promyelocytic Leukemia”, Hematology and Oncology, 2007

Lecturer – M1, “Epigenetics and Cancer”, OSU School of Medicine, 2007

Preceptorship, Department of Internal medicine, 2008

Preceptorship (Med II Hospital Based), Department of Internal Medicine, 2009

Preceptorship (CAPS), 1st year medical student, Department of Internal Medicine, 2010

Lecturer – “Epigenetics”, Med Student Lecture, 2010

Lecturer – “Epigenetics”, Med Student Lecture, 2011

Didactic, Selected lectures (partial list, truncated after 2014)

b. Graduate Programs:

i. Residency Programs:

Inpatient Resident, 2003-present

Ambulatory Resident, 2003-present

Continuity Clinic Resident Preceptor, 2003-present

Lecturer – “Acute Myeloid Leukemia”, Radiation Oncology Residents, 2005

Lecturer – “Hematologic Malignancies in the ICU”, Pulmonary Critical Care, residents, 2007

Lecturer – “Acute Leukemia,” Internal Medicine Residents Lecture, 2007

Lecturer – “Diagnosis and Treatment of Acute Leukemia,” Internal Medicine Resident Lecture Series, 2009

Lecturer – “Acute Myeloid Leukemia,” Internal Medicine Residents, 2014

List truncated beyond 2014

ii. Fellowship Programs:

Inpatient Fellow, 2003-present

Ambulatory Fellow, 2003-present

Continuity Clinic Fellow Preceptor, 2003-present

Lecturer – “Acute Myeloid Leukemia”, Radiation Oncology Fellows, 2005

Lecturer – “Hematologic Malignancies in the ICU”, Pulmonary Critical Care, fellows, 2007

Lecturer – “AML,” Hematology/Oncology Fellows Lecture, 2010

Lecturer – “High Risk AML,” Hematology/Oncology Fellows Lecture, 2010

Lecturer – “AML,” Hematology/Oncology Fellows Lecture, 2010

Lecturer – “Acute Myeloid Leukemia,” Hematology/Oncology Fellows Lecture, 2012

Lecturer – “Acute Lymphoblastic Leukemia,” Hematology/Oncology Fellows Lecture, 2013

List truncated beyond 2014, continued annual teaching of fellows.

iii. Master's and PhD Programs:

Lecturer – “Acute Leukemia”, Host Defense Lecture Series, 2007

Lecturer – Integrated Pathway Program (Medical Sciences 662), 2007

Lecturer – “Retinoids in hematologic malignancies”, Retinoid Study Group, 2007

Lecturer – “Acute Leukemia”, Host Defense Lecture Series, 2007

Lecturer - “Acute Myeloid Leukemia: Prognosis, Treatment, and the Role of Transplantation”, Genzyme Preceptorship Presentation, 2007

Lecturer – “Acute Leukemia”, Host Defense Lecture Series, 2007

Lecturer – “Lymphomas: How has molecular science affected patient care”, Cancer Immunology Lecture Series, 2009

Lecturer – “Acute Leukemias” Med I Host Defense Lecture Series, 2008

Lecturer – “How to interact ethically and productively with industry in clinical trials for cancer patients,” OSU Undergraduate College of Medicine, School of Biomedical Science (BMS 583) (approximately 15 OSU undergraduate students), 2009

Lecturer – “Acute Leukemias: From Bench to Bedside”, Cancer Immunology Series (approximately 20 post-graduate Ph.D. students), 2009

Lecturer – “Non-Hodgkins Lymphomas: From Bench to Bedside”, OSU School of Medicine, Cancer Immunology Course Series (approximately 20 post-graduate Ph.D. students), 2009

Lecturer – “Leukemia/Lymphoma,” IBGP Module 9 Cancer Biology. OSU College of Medicine, 2010, 2011

Lecturer – IBGP 880.02-SPTS- OSU College of Medicine, 2012

19. Supervisory Teaching:

Jason Chandler, 2008-2009, Private practice, Memphis, TN.

Alison Walker, 2009-2017, Associate Professor, Ohio State University

Jason Mendler, 2010-2011, Associate Professor, University of Rochester

Karilyn Larkin, 2013-2017, Assistant Professor, Ohio State University

Sumithira Vasu, 2013-present, Assistant Professor, Ohio State University

Tina Bhatnagar, 2014-2017, Assistant Professor, Ohio State University

Alice Mims, 2015-present, Assistant Professor, Ohio State University

Shylaja Mani, 2016-2017, Private Practice, Adena, OH

Geetika Bhatt, 2016-2017, Private Practice, Beachwood, OH

Colin Vale, 6/2020 to present, faculty at Emory as of 9/1/2022

20. Lectureships, Seminar Invitations, and Visiting Professorships:

a. National and International:

“MDS” Visiting Professorship, Lectures for Army Hematology/Oncology Fellows, Walter Reed Army Medical Center, Washington, DC, 2007.

21. Invitations to National/International, Regional, and Institutional Conferences:

b. National/International

"Diagnostic testing- acute leukemia and other hematologic malignancies: What do I send this for?" Transplant Nursing Conference, Bone Marrow Transplant Tandem Meeting Keystone, CO, February 7-9, 2007

“AML in the older adult: disease overview and current research”, Oncology Nursing Society (ONS) Congress, Las Vegas, NV, April 25, 2007

“Treatment of acute promyelocytic leukemia”, Oncology Nursing Society (ONS) Congress, Las Vegas, NV, April 25, 2007

“Epigenetic therapy in AML: Histone deacetylase inhibitors”, Clinical Advances in Epigenetic Therapy Conference, Charleston, SC, September 9, 2007

“Challenging cases in hematology, aggressive MDS”, Network for Medical Communication and Research, Las Vegas, NV, September 29, 2007

“Role of epigenetic therapy in AML”, New York Chemotherapy Foundation Symposium XXV, New York, NY. November 6-10, 2007

“Translational oncology: Pathways to future cancer therapies” Network for Oncology Communication and Research (NOCR) Annual Conference., Las Vegas, NV, March 7-8, 2008

“Tanespimycin-Bortezomib phase I trial in leukemia” Proteasome Inhibitor Combination Trials with Targeted Agents Meeting, National Cancer Institute, Bethesda, MD, April 18, 2008.

“Bortezomib as an epigenetic targeting agent”, Proteasome Inhibitor Combination Trials with Targeted Agents Meeting, National Cancer Institute, Bethesda, MD, April 19, 2008

“How to get an R21 Grant” American Society of Clinical Oncology International Meeting, Orlando, FL, June 2008

“CALGB 10503 Update and Future Plans”, CALGB Leukemia Committee Meetings, 2008-2012 (multiple lectures)

“Development of a new strategy for treatment of older AML patients”, CALGB Leukemia Core Committee Meeting, Chicago, IL September 13, 2009

“R21 Grants, Perseverance, and Resubmission”, Grant Writing Workshop I: Basic Grant Writing, American Society of Clinical Oncology Annual Meeting, Chicago, IL, June 2009.

“R21 Grants, Perseverance, and Resubmission”, Grant Writing Workshop I: Basic Grant Writing, ASCO Annual Meeting, Chicago, IL, June 2010

“Phase I trial of decitabine and bortezomib in AML”, CTEP Early Drug Development Meeting, Bethesda, MD, September 27, 2010

Chair, Education session for MDS, American Society of Hematology Annual Meeting, invited speaker for same session, “How Much, How Frequent, How Long? A Clinical Guide to New Therapies in MDS” Myelodysplastic Syndromes, Education Program, 2010 ASH Annual Meeting, Orlando, FL, December 4-5, 2010

“Myelodysplastic Syndromes”, 2011 Highlights of ASH in North America Meeting, Vancouver, BC, Canada, January 21, 2011

“Myelodysplastic Syndromes” 2011 Highlights of ASH in North America Meeting. New York, New York. February 4, 2011.

“Acute Myeloid Leukemia in Older Adults”, presentation as External Advisor to Eisai, Inc; Oncology Drug Advisory Committee to the US FDA, Consideration of Dacogen for acute myeloid leukemia, FDA White Oak Campus, Silver Spring, MD, February 9, 2012

“MDS and AML: an expert discussion panel”, Medscape. Online programming for CME. Cleveland, OH, September 2012

“How I Treat: Bringing Science to Clinical Dilemmas: Acute Myeloid Leukemia, 2013 ASH Annual Meeting, New Orleans, LA, Dec, 2013

Speaker, “Meet the Expert”, American Society of Hematology Annual Meeting 2013, New Orleans, LA

Invited Speaker, Debate Topic: Are Hypomethylating Agents the Standard of Care for Older Adults with AML Not Candidates for Intensive Chemotherapy? Society of Hematologic Oncology Meeting (SOHO), Houston, TX, Sept 18, 2014

AML Therapy – State of the Art, ImmunoGen, Inc. New York, Nov 19, 2014

“Novel Therapies in AML: Hope or Hype?” American Society of Clinical Oncology Annual Meeting, Chicago, IL, May 2014

International Conference on Acute Myeloid Leukemia, Molecular and Translational Advances in Biology and Treatment: Hypomethylating agents, European Society of Hematology, Budapest, Hungary, October 17, 2015

Acute Leukemia Forum 2016, Newport Beach, CA, “Why the stagnation in therapy for MDS?”, March 17, 2016

Pfizer Pharmaceuticals, “AML in 2017”, Phoenix, AZ, April 2017

“What’s new in myeloid malignancies”, Ion Solutions National Meeting, Nov 2018

ASH Review for Acute Leukemia, 2019 Multiple Myeloma Summit & ASH Review Meeting, Ion Solutions, Orlando, FL. Jan 26, 2019

“What’s new in myeloid malignancies”, Ion National Meeting, Nov 2019

Incorporating targeted agents into AML therapy, OncLive, Nov 2019

ASH Review for Acute Leukemia, 2020 Multiple Myeloma Summit & ASH Review Meeting, Ion Solutions, Orlando, FL. Jan 2020

“Advances in myeloid disorders this year”, Ion Oncology Practice Network National Annual Meeting, Nov 2020

“Advances in myeloid disorders this year”, Ion Oncology Practice Network National Annual Meeting, Nov 2021

Invited speaker, Meet the Professor ticketed session, Tandem Transplant Meetings, February 16, 2023, “Novel treatments in AML, who and when to transplant”.

c. Regional Meetings:

“Treatment of AML” a comprehensive overview of AML pathophysiology, screening, and diagnostic procedures, Nurse practitioners, pharmacists and fellows, Columbus, OH, November 14, 2005

“Acute myeloid leukemia and myelodysplastic syndrome”, Recognizing Blood Cancers in Family Practice, Leukemia and Lymphoma Society, Columbus, OH, May 13, 2006

“Myelodysplastic syndromes” Exciting new developments in hematology, The Ohio State University, Columbus, OH, May 26, 2006

“Epigenetic therapy in the treatment of myelodysplastic syndromes,” Cancer Center Grand Rounds, William Beaumont Cancer Center, Royal Oaks, MI, December 6, 2006

“Acute myeloid leukemia” Leukemia and Lymphoma Society, Columbus, Ohio, October 28, 2006

“Flavopiridol in acute leukemia” Examples of collaborative use of PK/PD cores & protocol development, The Ohio State University Pediatric Oncology Program Retreat, The Ohio State University, Columbus, OH, December 1, 2006

“A comprehensive overview of myelodysplastic syndrome pathophysiology, screening, and diagnostic procedures”, Nurse practitioners, pharmacists and fellows, Columbus, OH, March 19, 2007.

“The use of hypomethylating agents in treating aggressive MDS”, Akron community oncologists/Summa Health Systems practitioners, Indianapolis, IN, March 20, 2007

“Emerging therapies for MDS” Akron Community Oncologist and Practitioners, Akron, OH, May 15, 2007

“AML/MDS update”, Oncology and Hematology 2007: Review and Interpretation of the 2007 ASCO, ASH, AACR and other Major International Meetings, The Ohio State University, Columbus, OH, June 16, 2007

“Decitabine, a new therapeutic option for MDS”, Community Oncologist and Practitioners, Dayton, OH, July 9, 2007

“Myelodysplasia”, ASH Review, Columbus, OH, January 11, 2008

“Myelodysplastic Syndromes: An Update from ASH”, Leukemia & Lymphoma Society, CCONS, Columbus, OH, January 30, 2008

“Advances in management of myelodysplastic syndrome”, Grand Rounds, Western Kentucky Medical Center/ Commonwealth Health Corporation, Bowling Green, KY, August 14, 2008

“Advances in management of myelodysplastic syndrome”, Grand Rounds, Parkview Hospital, Fort Wayne, IN, September 15, 2008

AML/MDS and Transplantation: Genzyme Preceptorship Presentation, The Ohio State University, Columbus, OH, October 10, 2008

“Advances in the Management of Myelodysplastic Syndrome” Grand Rounds, Good Samaritan Hospital, Zanesville, OH, December 18, 2008

“Acute Leukemia” ASH Annual Conference Review, Columbus, OH, January 9, 2009

“Myelodysplastic Syndromes”, OptumHealth Education Program, Lebanon, OH, March 26, 2009

“Myelodysplastic Syndromes”, OptumHealth Education Program, Joliet, IL, April 2, 2009

“Myelodysplastic Syndromes”, OptumHealth Education Program, Ohio Cancer Specialist Inc. Mansfield, OH, April 29, 2009

“High-risk Myelodysplastic Syndromes”, OptumHealth Education Program, Columbus CCOP (approximately 30 community physicians and nurses), Columbus, OH, September 16, 2009

“Acute Leukemias” ASH Annual Conference Review, Columbus, OH, January 22, 2010

“Remissions in older AML patients treated with a novel schedule of Decitabine: miR-29b as a potential predictive marker”, OSUCCC Annual Scientific Meeting, Columbus, OH, February 19, 2010

“Advances in the Management of High-Risk AML”, Atlanta, GA, April 20, 2010

“Leukemia/Myeloma” Columbus CCOP 2010 ASCO Review, Columbus, OH, June 24, 2010

“Blood Disorders that cause cytopenias”, OSU MedNet 21 CME Webcast, Columbus, OH, July 1, 2011

“Myelodysplastic syndromes: a case based review”, Charles River Oncology Group, Framingham, MA, September 2012

“Myelodysplastic syndromes: a case based review”, Lima Oncology Group, Lima, OH, September 2012

“Novel Therapies in AML;”, Hematology Grand Rounds, MD Anderson Cancer Center, Houston, TX, April 9-10, 2013

“Misery and Joy, and Joy and Misery: Clinical Research in AML”, Invited lecture, University of NC, Nov, 2013

“Misery and Joy, and Joy and Misery: Clinical Research in AML”, Invited lecture, Yale University, Feb, 2014

“Clinical Trials in AML” Hematology Grand Rounds, UPMC, Pittsburgh, PA, April 6, 2016

“Use of Platelets in Clinical Leukemia and Transplantation Care”, Annual Red Cross Appreciation Day for Platelet Apheresis Donors, Columbus, OH, July 16, 2016

“Why Renee Canning is so great, and other stories”, (purpose-- CTO staff education and inspiration), Columbus, OH, July 15, 2016

“Acute leukemia and MDS”, OSU ASH Review, Columbus, OH, January 20, 2017

“Clinical research in AML”, Division of Hematology, Emory University, February 2017

“Beat AML,” Emory Sea Island Retreat for Hematology and Oncology, July 2017

“Novel approaches to treating AML in older patients”, Kennestone Hospital Heme/Onc Grand Rounds, Marietta, GA, Sept 2017 and Sept 2019

Duke Debates in Hematology. Blinatumomab vs Inotuzumab. Asheville, NC, April 14, 2018

Duke Debates in Hematology. Vyxeos vs Mylotarg. Asheville, NC, April 14, 2018

Lecture to Board of Directors for Georgia Chapter of the Leukemia & Lymphoma Society on acute myeloid leukemia, February 11, 2019

“Targeted therapies in AML,” Emory Sea Island Retreat for Hematology and Oncology, July 2018

“Immune based therapies are best in AML,” debate vs Hagop Kantarjian of MD Anderson who had the “Targeted therapies are best” part of the debate, Emory Sea Island Retreat for Hematology and Oncology, July 2018,

“Novel therapies in AML,” Emory Sea Island Retreat for Hematology and Oncology, July 2019

“New drugs in leukemia,” Leukemia and Lymphoma Society Annual Meeting (Georgia Chapter), 2019

Consenting a commercial CAR T patient, lecture to Janseen Pharm team, 3/31/21

TP53 mutations and myeloid malignancy, I had “no” part of the debate regarding allogeneic transplant. Emory Sea Island Retreat for Hematology and Oncology, July 2021

Maintenance therapy in AML. Emory Sea Island Retreat for Hematology and Oncology, July 2022

CART vs Allo Transplant debate, I had “CART” part of the debate. Emory Sea Island Retreat for Hematology and Oncology, July 2022

22. Abstract Presentations at National/International, Regional, and Institutional Conferences:
Hundreds of abstracts primarily at National/International meetings, not enumerated herein

23. Research Focus:

I develop novel therapies for patients with AML, especially those patients who older and unlikely to benefit from intensive chemotherapy. My focus is the introduction of novel treatments earlier into the course of treatment, with goal to expand use and access to allogeneic transplantation for older adults with AML.

24. Grant Support:

- a. Active Support:

i. Federally Funded: none active

ii. Contracts:

See Emory contracts support for active clinical trial support

- b. Previous Support:

Co-PI, NIH, 1R01CA188269-01, R01, \$207,500, 07/01/2014-06/30/2017

Clinical Protocol Leader, NCI, OSU Comprehensive Cancer Center Support Grant, P30, \$2,945,931, 12/1/2015-6/30/2017

PI, Boehringer Ingelheim Pharmaceuticals Inc, BI 836858, \$231,967, 09/11/2012-05/11/2019

Co-Investigator, Bristol-Myers Squibb Co, Fellowship and Clinical Trial Support-Collaborative Research Agreement between the OSUCCC and Bristol-Myers Squibb for Immuno-Oncology Studies, BMS/CCC Collaboration Agreement, \$651,544, 12/16/2015-12/16/2019

PI, BeGenBio, BGBC003, \$11,183, 02/15/2017-02/15/2019

PI, American Cancer Society Inc., Seed Grant, IRG-67-003-41, \$15,000, 10/31/2004

PI, Cell Therapeutics Inc., OSU0387 ARSENIC TRIOXID, \$17,126.13, 02/15/2006

PI, Cephalon Inc., OSU0438 PROTOCOL ATO101, \$3,185, 05/19/2006

Co-PI, National Cancer Institute, R01 CA102031, \$586302.91, 01/31/2010

Project Director for Project 4, National Cancer Institute, P50 CA140158, \$278,842, 07/31/2010

Co-PI, Astex Pharmaceuticals, Inc., SGI-110-01/OSU11013 , \$965,739, 10/13/2011-12/31/2017

PI, ImClone Systems Incorporated, CP17-0801, \$81,786, 10/31/2011

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PI, Celgene Corp, OSU 07095, \$144,203.22, 06/30/2012

PI, National Cancer Institute, R21CA128377, \$660,000, 04/30/2013

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APPENDIX B

APPENDIX B - MATERIALS CONSIDERED

EXHIBITS

No.	Exhibit
1001	U.S. Patent No. 8,846,628 (“’628 patent”)
1004	WO 2004/082619 (“Ionescu”)
1005	WO 2004/103358 (“Atadja”)
1006	Gibson, M., <i>Ch. 11, Oral Solid Dosage Forms</i> , PHARMA. PREFORMULATION AND FORMULATION, CRC Press (2001) (“Gibson”)
1007	Vidaza Label 2004
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1010	Press Release, Pharmion, <i>Clinical Data Presented on Pilot Pharmacokinetic Study of Oral Azacitidine</i> (June 2, 2007) (“Pharmion-PR”)
1011	Press Release, Pharmion, <i>Pharmion’s Oral Azacitidine Granted Fast Track Status for Myelodysplastic Syndromes</i> (August 29, 2007) (“Pharmion-PR II”)
1014	Chan et al., <i>5-Azacytidine Hydrolysis Kinetics Measured by High-Pressure Liquid Chromatography and 13C-NMR Spectroscopy</i> , 68(7) J. PHARMA. SCIS. 807 (1979) (“Chan”)
2003	Aparicio, A. & Weber, J., <i>Review of the clinical experience with 5-azacytidine and 5-aza-2’-deoxycytidine in solid tumors</i> , 3 CURRENT OPINION IN INVESTIGATIONAL DRUGS 627–633 (2002) (“Aparicio”)
2004	Beisler, J., et al., <i>Chemistry of Antitumor Triazine Nucleosides. An Improved Synthesis of Dihydro-5-Azacytidine</i> , 4(5) J.

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	CARBOHYDRATES NUCLEOSIDES NUCLEOTIDES 281–99 (1977) (“Beisler”)
2012	Čihák, A & Šorm, F., <i>Biochemical Effects and Metabolic Transformations of 5-Azacytidine in Escherichia coli</i> , 30 COLLECT. CZECH. CHEM. COMMUN. 2091–2102 (1965) (“Čihák”)
2018	Kaminskas, E., et al., <i>FDA Drug Approval Summary: Azacitidine (5-azacytidine, Vidaza™) for Injectable Suspension</i> , 10 THE ONCOLOGIST 176–82 (2005) (“Kaminskas”)
2029	Stresemann, C. & Lyko, F., <i>Modes of action of the DNA methyltransferase inhibitors azacytidine and decitabine</i> , 123 INT. J. CANCER 8–13 (2008) (“Stresemann”)
2055	U.S. Food and Drug Admin., <i>FDA approves Onureg® (azacitidine tablets) for acute myeloid leukemia</i> (2020) (“FDA Approves Onureg®”)
2056	Canadian Product 200mg Onureg® Product Information
2057	Canadian Product 300mg Onureg® Product Information
2058	Canadian Product Monograph Onureg®
2059	European Commission Decision 17.6.21
2062	Onureg® Label
2064	ALTMAN, R. & SARG, M., CANCER DICTIONARY: REVISED EDITION (2000) (“Altman”)
2068	Bankhead, C., <i>Year in Review: Acute Myeloid Leukemia</i> , MedPage Today (Nov. 29, 2020), https://www.medpagetoday.com/hematologyoncology/leukemia/89909 (“Bankhead”)

No.	Exhibit
2071	Carter, J., et al., <i>Targeting multiple signaling pathways: the new approach to acute myeloid leukemia therapy</i> , Signal Transduction and Targeted Therapy 5:288 (2020) (“Carter”)
2072	Choi, Y., et al., <i>Oral Maintenance Chemotherapy with 6-Mercaptopurine and Methotrexate in Patients with Acute Myeloid Leukemia Ineligible for Transplantation</i> , KOREAN ACADEMY OF MED. SCI., 30:1416–22 (2015) (“Choi”)
2073	Cogle, C., et al., <i>Oral Azacitidine (CC-486) for the Treatment of Myelodysplastic Syndromes and Acute Myeloid Leukemia</i> , 20 Oncologist 1404–12 (2015) (“Cogle”)
2075	CRAIG, C. & STITZEL, R., MODERN PHARMACOLOGY WITH CLINICAL APP. (6th Ed. 2004) 48–55 (“Craig”)
2076	Curtiss, F., <i>Pharmacy Benefit Spending on Oral Chemotherapy Drugs</i> , 12 J. OF MANAGED CARE PHARMACY 570–77 (2006) (“Curtiss”)
2080	Ellison, R., et al., <i>Arabinosyl Cytosine: A Useful Agent in the Treatment of Acute Leukemia in Adults</i> , 32 J. OF HEMATOLOGY 507–23 (1968) (“Ellisson”)
2081	The ASCO Post Staff, “FDA Approves Oral Azacitidine as Maintenance Therapy for Adults With AML in First Remission” (Sept. 25, 2020), https://ascopost.com/issues/september-25-2020/fda-approves-oral-azacitidine-as-maintenance-therapy-for-adults-with-aml-in-first-remission/ (“FDA Approves Oral Azacitidine”)
2082	Frisch, A., et al., <i>How we treat older patients with acute myeloid leukaemia</i> , 191 BRITISH J. OF HAEMATOLOGY 682–91 (2020) (“Frisch”)
2084	Garcia-Manero, G., et al., <i>Phase I Study of Oral Azacitidine in Myelodysplastic Syndromes, Chronic Myelomonocytic Leukemia, and Acute Myeloid Leukemia</i> , 29 J. OF CLIN. ONCOLOGY (2011) 2521–27 (“Garcia-Manero”)

No.	Exhibit
2086	Griffiths, E., <i>Oral hypomethylating agents: beyond convenience in MDS</i> , HEMATOLOGY (2021) (“Griffiths”)
2087	Grønbæk, K., et al., <i>Epigenetic Changes in Cancer as Potential Targets for Prophylaxis and Maintenance Therapy</i> , 103 BASIC & CLINICAL PHARM. & TOXICOLOGY 389–96 (2008) (“Grønbæk”)
2089	Kantarjian, H., et al., <i>Acute Myeloid Leukemia: Historical Perspective and Progress in Research and Therapy Over 5 Decades</i> , 21 CLIN. LYMPHOMA, MYELOMA AND LEUKEMIA 580–97 (2021) (“Kantarjian”)
2091	Kipp, D. & Wei, A., <i>The path to approval for oral hypomethylating agents in acute myeloid leukemia and myelodysplastic syndromes</i> , 17(20) FUTURE ONCOL. 2563–71 (2021) (“Kipp”)
2092	Kornblith, A., et al., <i>Impact of Azacytidine on the Quality of Life of Patients With Myelodysplastic Syndrome Treated in a Randomized Phase III Trial: A Cancer and Leukemia Group B Study</i> , 20 J. OF CLIN. ONCOLOGY 2441–52 (2002) (“Kornblith”)
2093	Lammers, P. & Cashen, A., <i>Azacitidine: A Review of its Use in the Management of Myelodysplastic Syndromes</i> , 1 CLIN. MED. THERAPEUTICS 1189–97 (2009) (“Lammers”)
2094	Lin, R., <i>Allogeneic Transplantation for Older Patients with Acute Myeloid Leukemia: The Dawn of a New Era</i> , 28 TRANSPLANTATION AND CELLULAR THERAPY 793–94 (2022) (“Lin 2022”)
2095	Liu, H., <i>Emerging agents and regimens for AML</i> , J. HEMATOLOGY & ONCOLOGY 14:19 (2021) (“Liu”)
2097	Löwenberg, B., et al., <i>Mitoxantrone Versus Daunorubicin in Induction-Consolidation Chemotherapy</i> , 16 J. OF CLIN. ONCOLOGY 872–81 (1998) (“Löwenberg”)
2098	Ma, X., et al., <i>Myelodysplastic Syndromes: Incidence and Survival in the United States</i> , 109 AM. CANCER SOC. 1536–42 (2007) (“Ma”)

No.	Exhibit
2102	Odenike, O., <i>Incorporating novel approaches in the management of MDS beyond conventional hypomethylating agents</i> , Myelodysplastic Syndrome: Understanding the Current Treatment Landscape 460–69 (2017) (“Odenike”)
2103	Goodman, A., “Oral CC-486 Maintenance Therapy Extends Survival in Older Patients with AML” Today in Oncology (Jan. 25, 2020), https://ascopost.com/issues/january-25-2020/oral-cc-486-maintenance-therapy-extends-survival-in-older-patients-with-aml/ (“Oral CC-486”)
2104	Perl, A., <i>The role of targeted therapy in the management of patients with AML</i> , Blood Advances and Hematology 2281–94 (2017) (“Perl”)
2108	Preisler, H., et al., <i>Comparison of Three Remission Induction Regimens and Two Postinduction Strategies for the Treatment of Acute Nonlymphocytic Leukemia</i> , 69 BLOOD 1441–49 (1987) (“Preisler”)
2110	Récher, C., <i>The beginning of a new therapeutic era in acute myeloid leukemia</i> , 2 J. HAEMATOLOGY 823–33(2021) (“Récher”)
2111	Reville, P., <i>Maintenance Therapy in AML</i> , 10 FRONTIERS IN ONCOLOGY (2021) (“Reville”)
2114	Slater, H., <i>Phase 3 QUAZAR AML-001 Trial Finds Oral Azacitidine Extends Survival in Older Adults with AML</i> , Cancer Network (Jan. 12, 2021) (“Slater”)
2115	Sochacka-Ćwikła, A., et al., <i>FDA-Approved Drugs for Hematological Malignancies—The Last Decade Review</i> , CANCERS (2022) (“Sochacka-Ćwikła”)
2118	Sorrentino, V., et al., <i>Hypomethylating Chemotherapeutic Agents as Therapy for Myelodysplastic Syndromes and Prevention of Acute Myeloid Leukemia</i> , 14 PHARMACEUTICALS 641(2021) (“Sorrentino”)

No.	Exhibit
2119	Southall, J., “Oral azacytidine extends survival for patients with AML in remission after chemotherapy” HemOncToday (Jan. 5, 2021) (“Southall”)
2120	Steensma, D., <i>ASH 2010 Meeting Report—Top 10 clinically-oriented abstracts in myelodysplastic syndromes (MDS)</i> , Am. J. of Hematology 385–91 (2011) (“Steensma”)
2125	Vigil, C., et al., <i>Safety and efficacy of azacitidine in myelodysplastic syndromes</i> , 4 DRUG DESIGN, DEVELOPMENT AND THERAPY 221–29 (2010) (“Vigil”)
2129	Wei, A., et al., <i>Long-term survival with oral azacitidine for patients with acute myeloid leukemia in first remission after chemotherapy</i> , 98 AM. J. OF HEMATOLOGY E84–87 (2023) (“Wei”)
2130	Weill Cornell Medicine, “New maintenance treatment for acute myeloid leukemia prolongs the lives of patients” NEW ENGLAND J. OF MED. (Jan. 22, 2021), https://news.weill.cornell.edu/news/2021/01/new-maintenance-treatment-for-acute-myeloid-leukemia-prolongs-the-lives-of-patients , (“Weill Cornell”)
2133	Exemestane Label (“Exemestane”)
2134	Anastrozole Label (“Anastrozole”)
2135	Letrozole Label (“Letrozole”)
2136	Blum W, et al., <i>Maintenance therapy with decitabine in younger adults with acute myeloid leukemia in first remission: a phase 2 Cancer and Leukemia Group B Study (CALGB 10503)</i> , 31 LEUKEMIA 34-39 (2017) (“Blum”)
2137	Cytarabine Label (“Cytarabine”)
2138	Dacogen Label (“Dacogen”)
2139	Mitoxantrone Label (“Mitoxantrone”)

No.	Exhibit
2140	Finkel, M., <i>The Orphan Drug Act and the Federal Government's Orphan Products Development Program</i> , 99 PUBLIC HEALTH REPS. 313–16 (1984) (“Finkel”)
2141	Martinez, M. & Amidon, G., <i>A Mechanistic Approach to Understanding the Factors Affecting Drug Absorption</i> , 42 J. OF CLIN. PHARMACOLOGY 620–43 (2002) (“Martinez”)
2142	European Medicines Agency, Assessment Report of Onureg EMA/308711/2021 (“European Medicines Report”)
2150	Huls, G., et al. <i>Azacitidine maintenance after intensive chemotherapy improves DFS in older AML patients</i> , 133 BLOOD, THE JOURNAL OF THE AMERICAN SOCIETY OF HEMATOLOGY 1457–64 (2019) (“Huls”)