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PROTECTING INNOVATION IN RARE CANCER

REBALANCE THE IRA

PIRC Meeting

May 5, 2025

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PIRC Purpose

- Speak loudly with one voice
- Provide **a rare cancer perspective**
  - *Educate ourselves (& our communities)*
  - Applaud what we can
  - Prepare for what is coming
  - Fight against what must change
    - NOW AND NEXT YEAR
    - PREPARATION

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Agenda

- Calquence Roundtable (April 17)
- Town Hall (April 30)
- Follow Ups/Next Steps
- Relevance to Rare Cancers Generally

Context: Round II…

Round II

- 15 Part D Drugs Selected
- Single MFP, subject to an MFP ceiling, across all dosage forms and strengths, based on 30-day supply

- Timeline
  - Feb 1‘25: Deadline for CMS to publish the list of 15 Part D drugs for negotiation
  - Feb 28’25: Deadline for manufacturers of selected drugs to sign negotiation agreements.
  - March 1 ’25” Deadline for manufacturers to submit required data
  - Post-March 1, ‘25: Patient-focused listening sessions and other opportunities to engage with CMS
  - June 1’25: CMS to send initial offers to manufacturers.
  - July 1’25: Manufacturers to accept/reject initial offer/propose a counteroffer
  - Summer ‘25: CMS to respond to counteroffers
  - October 31 ‘25: Manufacturers to accept/reject CMS’s final offer
  - November 30 ‘25: CMS to publish final prices for 2027
  - March 1’26: CMS to publish an explanation of final prices

“Listening Sessions” Redefined

CMS will also host patient-focused *events* to seek verbal input from patients and other interested parties.
“Events” intended to bring together patients, beneficiaries, caregivers, and consumer and patient organizations, other interested parties to share patient-focused feedback with CMS on patient experiences with the conditions or diseases treated by the drug/alternatives as CMS reviews data submissions and develops an initial offer for each selected drug.

“CMS intends to improve upon the design of the patient-focused listening sessions …”

- Solicited comments on event format, scope, and logistics. ….
- CMS considering “discussion” among speakers and in which CMS may ask clarifying questions
- CMS weighing different event formats…solicited comments on ways to promote discussion

Instead of livestreaming, CMS considering publishing an event summary/redacted transcript

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From last year, recall factors CMS Will Continue to Consider …

CMS reiterates it considers "alternative treatments" in setting initial and final price:

1. Extent to which selected drug represents a therapeutic advance compared to existing therapeutic alternatives for the selected drug and the costs of such existing therapeutic alternatives;

2. FDA-approved prescribing information for the selected drug and its therapeutic alternatives;

3. Comparative effectiveness of the selected drug and its therapeutic alternatives, including the effects of the selected drug and its therapeutic alternatives on specific populations (incl/ individuals with disabilities, elderly, terminally ill, children, and other patient populations); and

4. Extent to which the selected drug and the therapeutic alternatives to the drug address unmet medical needs for a condition for which treatment or diagnosis is not addressed adequately by available therapy

Patients/Patient Orgs Can Submit:

- ✓ information on the drugs, "alternatives," impact on specific sub-pops, patients experience, and unmet need
- ✓ Outcomes – e/g., changes to productivity, independence, quality of life considered when these correspond w/ direct impact on the individuals taking the drug/alternative if appropriately measurable and quantifiable;

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April 17 Calquence Roundtable -- Format

- Session was moderated by RTi, not CMS
- CMS was listening, but did not interact
- RTI moderator did not take information from participants on one question and use it to follow-up for the next question(s), so the discussion was not dynamic at all.
- Questions became repetitive and appeared to completely misunderstand CLL or any other rare cancer – Qs were for conditions with lots of treatment options, not a rare cancer with a lot of remaining unmet need.
- Before beginning, RTI asked participants to blur their backgrounds and remove last names; discussion was recorded but not publicly available
- “Discussion” in the sense (i) participants could build on what each other were saying, and (ii) the format did not limit people to a static 3 min. ‘speech’ -- so a bit more fluid and more comfortable for participants than last year.

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April 17 Calquence Roundtable -- Participants

CMS indicated “up to nine participants”
Email indicated three Qs would be asked:

- Patients’ day-to-day experiences living with the condition(s) treated by the selected drug, including how the experience may differ for different patient populations as well as patients’ caregivers and families.
- How the selected drug impacts patients, including both benefits and side effects, as compared to the therapeutic alternative(s), and which outcomes matter most to patients with the condition(s) treated by the selected drug.
- Any other information about the selected drug, the condition(s) it is used to treat, and other treatments used for that condition(s) that the speaker believes CMS should consider.

CLL Society organized the following patients/patient group perspectives:

- Diagnosed early, still watch and wait, expecting a long time living with CLL, cognizant of duration of options
- Several patients received their first treatment well before BTK-I’s were available and could describe experience with Imbruvica, including toxicities
- One patient received Calquence as initial therapy and has had access hurdles (PA denial) requiring appeals, mediation, etc., leading to missed doses
- Patient group perspective – policy issues but also reiterating implications of patient stories for CMS
- Responding to any other of the nine participants

April 17 Calquence Roundtable -- Format

CLL, when symptomatic, can have a profound impact on a person's life:

- Extreme fatigue, making it impossible to work, perform essential daily living tasks, etc.
- Pain association with swollen spleen, lymph nodes
- One person talked about inability to swallow due to swollen lymph nodes even though his "numbers" may not have triggered decision to start treatment
- Change in appearance due to lymphatic swelling
- Immunocompromised – impacts decisions on social commitments (can be isolating), travel, etc.
- *All "our" speakers underscored need to have ALL treatments readily accessible*
- Several patients said that if it were not for BTK inhibitors, they almost certainly would not be alive
- *Post-approval research is crucial.* New treatment protocols for fixed duration can be helpful in reducing time on treatment while maintaining progression free survival
- There is still enormous unmet need. Once a patient has moved through the available treatments, only option is to find a study that will accept them
- Insurance is still a problem. The *financial burden is no longer the biggest problem for patients*, but several patients spoke about getting denials and one said they had to go through mediation to get a 2nd year of treatment through PA. This goes against any and all CLL guidelines.
- Imbruvica was a gamechanger, but the side effects were very bad, and some people had to go off treatment. Calquence has a much better safety profile
- There was *some discussion about how the "pill penalty"* is going to *impact post-approval research*. This could be devastating for the cancer community.

April 30 Calquence Town Hall -- Format

- Unlike the Roundtable discussions, the two Town Halls were open to the public.
- The session was moderated by RTI and proceeded similarly to last year's listening sessions in that each speaker had a short statement and the moderator gave a "time's up; wrap up, etc." nudge.
- A Q&A period followed the statements for each of the selected drugs.
- Clinicians/researchers delivered statements during the Calquence portion of the Town Hall.
- The discussion was recorded but not yet publicly available.
- There was no interaction between among speakers.

In preparation, CMS provided the following optional topics:

•Experience prescribing and/or managing treatment with selected drug/therapeutic alternatives for the condition(s)

•Information re: goals of the treatment for the condition(s) treated by the selected drug, including outcomes used to assess improvement or treatment response

• Considerations that drive treatment choice among selected drug and therapeutic alternatives

• Information about extent to which the selected drug currently represents (or does not represent) a therapeutic advance as compared to therapeutic alternatives

• Information about the extent to which the selected drug or therapeutic alternatives currently address (or do not address) an unmet medical need for the condition(s) treated by
the selected drug.

• Any other information re: selected drug, the condition(s), other treatments

April 30 Calquence Town Hall -- Participants

Dr. Matthew Davids – leads Lymphoma program at the Dana-Farber/Harvard Cancer Center; Director of Clinical Research in the Division of Lymphoma at the Dana-Farber Institute; Associate Professor of Medicine at Harvard Medical School

Laura, Zitella, MS, RN, ACNP-BC, AOCN – nurse practitioner in Hematology at UCSF and associate clinical professor at School of Nursing in the Hematology, Blood and Marrow Transplant and Cellular Therapies Program at UCSF Comprehensive Cancer Center

Dr. Nicole Lamanna, Director of the Chronic Lymphocytic Leukemia Program, Hematologic Malignancies Section at the Herbert Irving Comprehensive Cancer Center at New York-Presbyterian/Columbia University Medical Center

Another Dartmouth clinician presented on behalf of ASH. The clinician talked about financial toxicities with these treatments and potential for negotiated price to reduce costs for patients. Dr. Lamanna was able to clarify that the Part D cap will relieve financial stressors for patients and negotiated price is unlikely to impact out-of-pocket costs.

April 30 Calquence Town Hall -- Discussion

Discussion

- CLL continues to be incurable but highly treatable
- Goal of therapy is to help people live as long as possible feeling as well as possible
- It was incredible advance in CLL care when the BTKi, Ibrutinib was developed and approved
- Ibrutinib has already been negotiated but has a lot of side effects (e.g., irregular heart rhythms, high blood pressure, joint pain, rash, and diarrhea). About 40% of patient stop therapy due to intolerable side effects
- The OOP cap in Part D will be a big help to Medicare patients. Affordability is a huge issue that affects people being able to stay on their life prolonging therapy
- Building on the effectiveness of BTK inhibitors as a single agent, we have started combining BTKs w/ other medications such as venetoclax. Acalabrutinib and venetoclax work better together than either alone, can result in a remission so deep that patients can stop treatment after a period of time
- Limiting the time on treatment decreases side effects, minimizes costs and decreases change of resistance to treatment
- Patient starting treatment generally favor time-limited treatment, and Acalabrutinib plus venetoclax are in the NCCN guidelines
- Data is not yet completed, but there may be opportunity to “re-use” Calquence w/ the fixed duration combination
- Different patients may need different drugs. Hypertension patients should be on Calquence, patients with serious headaches would generally be started on Brukinsa instead
- Sometime patients have side effects after they start one treatment and need to be quickly switched. Here, it is critical to have immediate access since missed doses can cause a disease flare

April 30 Calquence Town Hall -- Discussion

Discussion

- Mantle cell lymphoma is challenging to treat as it is also incurable but it is more aggressive than CLL. BTK inhibitors such as Calquence are very active against MCL and there is a new first line indication for Calquence in combination regimen
- A Dartmouth clinician appeared to have the same misconception several speakers had last year – i.e., that negotiated prices would result in lower OOP costs for patients.
- The Part D cap fixes the OOP problem and for higher cost drugs, the MFP will not impact patients' bottom line.

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Next Steps

Send meeting request to CMS
Reiterate points of discussion
Focus on patient org responsibility to inform patient community
Speak to OOP costs, treatment combinations and maintaining access to “all or substantially all”
Address pill penalty consequences and this Administration’s interest in addressing

NEXT PIRC CALL: PLS NOTE JUNE 9th 1pm ET