Step Therapy in Medicare Part D Oncology Drugs

Executive Summary

Escalating drug costs in the United States are placing pressure on both the federal government and health plans to implement strategies aimed at controlling drug expenditures and optimizing utilization. Utilization management (UM) tactics are frequently used in Medicare Part D, by both Medicare Advantage-Prescription Drug plans (MA-PDs) and standalone Prescription Drug Plans (PDPs), to control prescription drug spending. While UM is used to lower plan spending and premiums for beneficiaries, it can also make it more difficult for patients to access the prescriptions they need, especially high-cost oncology drugs.

To better understand how UM is used in Medicare, the American Cancer Society Cancer Action Network (ACS CAN) engaged Avalere to conduct an analysis to understand the extent to which step therapy restrictions exist for certain drugs that treat breast cancer, cyclin-dependent kinase 4 and 6 (CDK 4/6) inhibitors. The CDK 4/6 class includes four drugs: Ibrance, Kisqali, Kisqali Femara co-pack, and Verzenio. Results indicate there were few differences in formulary tiering and UM across the four drugs evaluated; however, there were differences in tiering and UM by plan type. Differences in tiering occurred within Special Needs Plans (SNPs), which placed the four drugs on the specialty tier 45% of the time and the preferred brand tier 55% of the time.[1] All non-SNP MA-PDs and all standalone PDPs placed the four drugs on the specialty tier 100% of the time.

While no plans explicitly require step therapy (ST) in the formulary design, Avalere evaluated the detailed restrictions policies to understand whether ST requirements were embedded within prior authorization (PA) requirements for all Part D plans. Many plans included ST embedded within their PA criteria, with step edits dependent on patient characteristics or treatment choice. Kisqali and Kisqali Femara co-pack included ST requirements embedded within PA requirements that were dependent on beneficiary characteristic or treatment choice 23% of the time, while Ibrance and Verzenio included ST requirements 23% and 82% of the time, respectively. For Ibrance, step therapy requirements were dependent on treatment type, and for Kisqali, Kisqali-Femara co-pack and Verzenio, step requirements were dependent on both patient characteristics and treatment type.

Introduction

Escalating drug costs in the United States are placing pressure on both the federal government and health plans to implement strategies aimed at controlling drug expenditures and optimizing utilization. UM tactics are frequently used in Medicare Part D by both MA-PDs and standalone PDPs to control prescription drug spending. While UM is used to lower plan spending and premiums for beneficiaries, it can also make it more difficult for patients to access the prescriptions they need, especially high-cost oncology drugs.

[1] SNPs provide the same Medicare Parts A and B coverage for beneficiaries as other MA plans but also offer tailored benefits and coordinated care for beneficiaries that have specific health conditions or meet other requirements.

The use of PA and ST in Part D and Medicare Advantage (MA) has come under increased scrutiny in recent years, with some stakeholders asserting that plans' use of PA inappropriately restricts beneficiary access to care. PA protocols are reasonable when they are beneficiary-centered and based on robust clinical evidence. However, when they limit patient access to medically appropriate drugs, they may adversely impact beneficiary health.

The implementation of the Inflation Reduction Act (IRA) includes changes to the Part D benefit design that require manufacturers and plans to pay an increased share of drug costs for Part D enrollees. Plans are taking on significantly more liability because of the IRA, particularly for beneficiaries receiving low-income subsidies and patients in the catastrophic phase of the benefit, which cancer patients are disproportionately more likely to reach compared to the average Part D beneficiary.[2] These changes in the benefit design give plans a greater incentive to control access to Part D-covered drugs through the use of UM.[3]

The shift in plan liability under the IRA increases the importance of the Part D risk adjustment model, which predicts plan liability based on the health status, medical diagnoses, and demographic characteristics of a plan's enrollees to adjust plan payments accordingly. However, concerns persist around the accuracy of the model in adjusting payments for certain conditions. For example, an Avalere analysis based on 2019 MA-PD and PDP data found that plan spending was underpredicted by 53% for oncology and by more than 60% for three other therapeutic areas, including autoimmune conditions, hepatitis C, and multiple sclerosis.[4] As Part D benefit design changes from the IRA are implemented, CMS's adjustments to the risk score model will be a significant factor in how Part D plans manage their increased liability, and subsequently the incentive to change beneficiary behavior using UM..[5]

The likelihood that IRA changes will increase plans' use of UM and other formulary restrictions underscores the importance of a thorough and robust formulary review process from CMS. CMS reviews formularies for coverage, tier placement, and utilization management, but there is limited public information on CMS's formulary review processes and findings. CMS also conducts outlier tests across each area of review based on formularies among other Part D plans and best practice formularies. These outliers are further evaluated by CMS to determine if the outlier is deemed potentially discriminatory. However, outlier tests only capture differences among plans and may not identify systemic issues across plans.

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[2] "Catastrophic Coverage in the Medicare Part D Drug Benefit: Which Beneficiaries Need It and How Much Are They Spending?" 2020. Commonwealth Fund. September 17, 2020. https://www.commonwealthfund.org/publications/issue-briefs/2020/sep/catastrophic-coverage-medicare-part-d-drug-benefit.

[3] Hayden. Inflation Reduction Act: Revisiting Price Negotiation & its Anticipated Impact on the Biopharma Landscape. January 2024. https://haydencg.com/wp-content/uploads/2024/01/IRA-Revisiting-Price-Negotiation-Paper.pdf.

[4] Avalere. Risk Adjustment Under Part D Benefit Redesign. February 27, 2023. https://avalere.com/insights/risk-adjustment-under-part-d-benefit-redesign.

[5] Carioto, Jennifer, Gabriela Dieguez, and Tushar Makhija. "Financial implications of the Inflation Reduction Act are expected to lead to a reassessment of formulary strategies by Part D plans," December 21, 2023. https://www.milliman.com/en/insight/financial-implications-ira-formulary-strategies-part-d.

Background on UM

UM refers to a variety of practices that health plans and pharmacy benefit managers use to manage the utilization of specific drugs or services. Plans can use UM to shift utilization between treatment options or to limit utilization of high-priced, specialty medications by ensuring that a prescribed drug or service is medically necessary. In practice, growing evidence suggests that UM strategies impede the delivery of timely, efficient, and high-quality care and can lead to delays in care, administrative burden, and worse outcomes.[6,7,8] For purposes of this study, UM policies are categorized into two categories: ST and PA, although other UM types exist, such as quantity limits.

Prior Authorization

PA is the requirement that a patient receive pre-approval from their health insurance company before a medical service or treatment can be covered or reimbursed. In a 2022 survey of American Society of Clinical Oncology members, nearly all respondents reported at least one of their patients has experienced harm because of prior authorization processes. Respondents reported harms including delays in treatment (96%), increased patient out-of-pocket costs (88%), denied therapy (87%), disease progression (80%), and even loss of life (36%). Notably, 64% of respondents reported that a patient abandoned care.[9]

Step Therapy

ST requires patients to try and fail (or "step through") an insurer's preferred medication before the patient can access the medication originally prescribed by their health care provider. Step therapy can delay access to proper treatments, which can result in potentially irreversible disease progression or other adverse outcomes.[10]

Impact of UM on Beneficiary Access

Although plans use UM to reduce costs and premiums for beneficiaries, a report from the U.S. Department of Health and Human Services Office of Inspector General suggests that UM policies create multiple potential access barriers for Medicare beneficiaries.[11] Medicare does allow for appeals of coverage decisions, in both a standard and expedited process. If an enrollee receives an unfavorable determination from the plan, they may request reconsideration

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6 "New Survey: Utilization Management Delays Cancer Care; Leads to More Stress and Contributes to Worse Outcomes." 2019. American Cancer Society Cancer Action Network. March 28, 2019. https://www.fightcancer.org/releases/new-survey-utilization-management-delays-cancer-care-leads-more-stress-and-contributes.

7 Park, Yujin, Syed Raza, Aneesh George, Rumjhum Agrawal, and John Ko. 2017. "The Effect of Formulary Restrictions on Patient and Payer Outcomes: A Systematic Literature Review." Journal of Managed Care & Specialty Pharmacy 23 (8): 893–901. https://doi.org/10.18553/jmcp.2017.23.8.893.

8 Tharp, Louis, and Zoe Rothblatt. "Do Patients Benefit from Legislation Regulating Step Therapy?" Health Economics, Policy and Law 17, no. 3 (2022): 282-97. doi:10.1017/S1744133121000153.

9 Association for Clinical Oncology. "ASCO Prior Authorization Survey Summary." ASCO. November 2022. https://old-prod.asco.org/sites/new-www.asco.org/files/ASCO-Prior-Auth-Survey-Summary-November-2022.pdf.

10 Ibid.

11 US Department of Health and Human Services. Office of Inspector General. *Some Medicare Advantage Organization Denials of Prior Authorization Requests Raise Concerns about Beneficiary Access to Medically Necessary Care*, by Christi A. Grimm. Washington, DC, April 2022. https://oig.hhs.gov/oei/reports/OEI-09-18-00260.pdf.

from the plan, followed by additional levels of appeal. Of concern, CMS audit data reveal that a small minority of beneficiaries use the appeals process.[12] Research on access to medication has found significant complexity in the Medicare appeals process, including cumbersome requirements for beneficiaries, compounded delays in treatment due to multiple layers of review, and a lack of information transparency around the use of Medicare appeals processes.[13]

Medicare Protected Classes

It is essential that beneficiaries with a severe, complex, and progressive condition like cancer have broad access to all available therapies so that patients' unique needs can be addressed, and to avoid preventable disease progression. To ensure beneficiaries with certain health issues receive necessary treatment, CMS requires that all Part D plans must include "all or substantially all" drugs in each of the six protected classes (i.e., anticonvulsants, antidepressants, antineoplastics, antipsychotics, antiretrovirals, and immunosuppressants) on their formularies.[14] The purpose of this policy is to ensure beneficiaries with certain conditions receive the treatment they require without fear of not having their treatment covered, though UM is still allowed for most drugs covered under a protected class.[15]

Project Overview

ACS CAN engaged Avalere to evaluate formulary and policy restrictions in Part D plans for certain oncology drugs, CDK 4/6 inhibitors, to understand whether plans are imposing ST restrictions on these products. CDK 4/6 inhibitors are used to treat certain types of hormone receptor-positive breast cancer. Avalere evaluated the plan year 2023 MA-PD and PDP formulary coverage and restrictions policies for four branded CDK 4/6 inhibitors. Avalere partnered with Clarivate™ to obtain formulary and restrictions data and pharmacy lives covered across all Medicare payer types.[16] Restrictions and policy analyses were limited to plans for the top MA-PD and PDP plan sponsors based on enrollment.

Methodology

Avalere evaluated the 2023 formulary coverage and restrictions policies for four branded CDK 4/6 inhibitors with no generic equivalents: Ibrance (palbociclib), Kisqali (ribociclib), Kisqali Femara co-pack (ribociclib and letrozole), and Verzenio (abemaciclib). Formulary analyses were based on all Part D plans (42.7 million member lives) while restrictions policy data were limited to Part D sponsors with at least 200,000 covered pharmacy lives. The restrictions information

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[12] Center for Medicare & Medicaid Services (CMS). Analysis of Calendar Year 2017 Medicare Part D Reporting Requirements Data. https://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovContra/PartCDDataValidation.html.

[13] "The Medicare Appeals Process: Reforms Needed to Ensure Beneficiary Access." 2020. American Cancer Society Cancer Action Network. November 17, 2020. https://www.fightcancer.org/policy-resources/medicare-appeals-process-reforms-needed-ensure-beneficiary-access.

[14] Centers for Medicare and Medicaid Services. *Medicare Prescription Drug Benefit* Manual Chapter 6 -Part D Drugs and Formulary Requirements. https://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovContra/Downloads/Part-D-Benefits-Manual-Chapter-6.pdf.

[15] UM is permitted for beneficiaries initiating therapy in all protected classes except for antiretrovirals.

[16] Certain data included herein are derived from the Fingertip Analytics© of Clarivate. All rights reserved.

was analyzed at a static point in November 2023; formulary changes over time were outside the scope of the analysis.[17]

In the restrictions analysis, Avalere first identified the plans requiring prior authorization for the CDK 4/6 inhibitors. For these plans, Avalere then parsed the criteria text to identify whether there was a step therapy edit required. The step edits require some beneficiaries to try other therapies prior to receiving approval for the CDK 4/6 inhibitor, have certain demographic characteristics, or reach a certain level of disease progression.

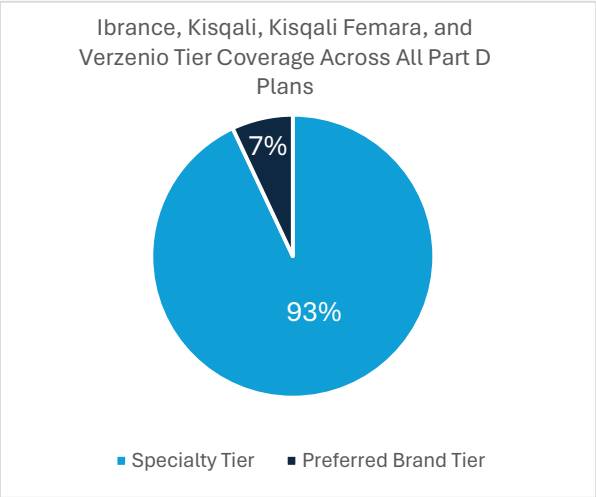
Results

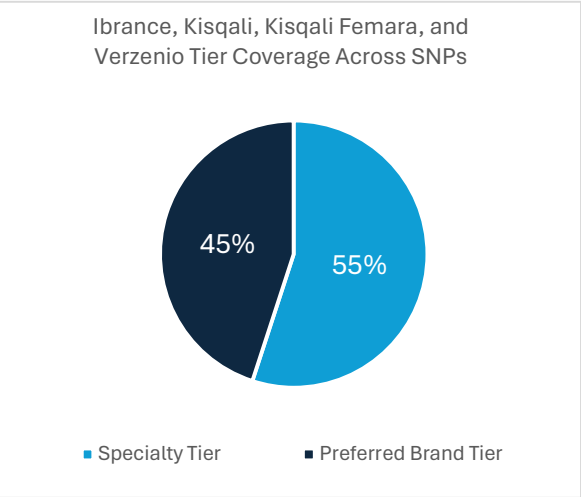
MA-PD and PDP CDK 4/6 Inhibitor Formulary Tiering

Because antineoplastics (drugs that stop tumor growth) are a protected class, the four CDK 4/6 inhibitors were covered 100% of the time by Part D plans. There were few differences in formulary tiering and UM across the four drugs evaluated, however there were differences in tiering and UM by plan type.[18]

Across all Part D plans, the four drugs were placed on the specialty tier 93% of the time, and the preferred brand tier 7% of the time (Figure 1). Differences in tiering occurred within the Medicare Advantage Special Needs Plans (SNPs), which placed the four drugs on the specialty tier 45% of the time and the preferred brand tier 55% of the time (Figure 1). All PDPs and non-SNP MA-PDs placed the four drugs on the specialty tier 100% of the time.

Figure 1: 2023 CDK 4/6 Inhibitor Overall Part D Plan and SNP Tiering





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[17] Plans can and do change their formularies over the course of the plan year. New plan data in 2023 may not be reflected in the data due to slow implementation of UM.
[18] No UM listed by a plan could be due to an error or lack of information from the Plan Sponsor.

Across all four drugs, Part D plans required prior authorization 97% of the time and had no UM requirements 3% of the time. All PDPs and 98% of SNPs required PA for the four drugs. Across MA-PDs, plans required PA 93% of the time for Ibrance and Verzenio, and required PA 94% of the time for Kisqali and Kisqali Femara co-pack.

MA-PD and PDP CDK 4/6 Inhibitor Step Therapy Restrictions

While no plans explicitly require ST in the formulary design, Avalere evaluated the detailed restriction policies in place to determine whether ST requirements were embedded within PA requirements for the largest Part D plan sponsors. In total, restrictions policies for 190 plans (36 PDPs, 113 MA-PDs, and 41 SNPs) were evaluated, representing 78% of covered lives under Part D.

The PA detail for the four drugs included multiple requirements or specifications for different conditions. For example, a plan may specify different requirements if the beneficiary is pre- or perimenopausal versus post-menopausal or based on the type of breast cancer (Table 1). As a result, there are cases where the prior authorization criteria may embed a step edit for a specific subset of the beneficiary population, but not all beneficiaries. In the case of a beneficiary with metastatic breast cancer seeking approval for treatment with Verzenio monotherapy, one example analyzed requires a beneficiary to step through endocrine therapy and chemotherapy first (Figure 2). Though "coded" in data as a prior authorization restriction, in practice, the patient would experience the requirement as step therapy.

Based on these nuances, Avalere evaluated the presence of step edits based on the interpretation that a step edit for any beneficiary (even if a subset of the beneficiary population) means that the PA criteria includes an embedded step edit.

Table 1: Examples of Step Edit Requirements Dependent on Patient Characteristic or Treatment Choice

Dependent on Patient Characteristic	• Required: Diagnosis of **advanced or metastatic HR-positive, HER2-negative breast cancer** and one of the following:
	  ○ The patient is a man or **pre-or perimenopausal** woman and the requested drug will be used in combination with an aromatase inhibitor as initial endocrine-based therapy,
	  ○ The patient is a man or **postmenopausal** woman, the requested drug will be used in combination with an aromatase inhibitor as initial endocrine-based therapy, and the patient has experienced disease progression, an intolerable adverse event, or contraindication to palbociclib or abemaciclib,
	  ○ The patient is a man or **pre-or perimenopausal** woman and the requested drug is being used with fulvestrant as initial endocrine-based therapy, or
	  ○ The patient is a man or **postmenopausal** woman, the requested drug is being used following disease progression on endocrine therapy, and the patient has experienced disease progression, an intolerable adverse event, or contraindication to palbociclib or abemaciclib.
	• Age Restrictions: **18 years of age and older**
	• Prescriber Restrictions: Prescribed by or in consultation with an oncologist.

Dependent on Treatment Choice	• Exclusion Criteria: Member has **experienced disease progression** on CDK 4/6 inhibitor (e.g., ribociclib, abemaciclib).
	• Required Medical Information: The member has a **diagnosis** of HR positive and HER 2-negative breast cancer AND one of the following applies:
	  ○ The member will be using Ibrance in **combination with an aromatase inhibitor** (e.g., letrozole) as initial endocrine-based therapy for their recurrent disease OR
	  ○ The member will be taking Ibrance in **combination with an aromatase inhibitor** (e.g., letrozole) as initial endocrine based therapy for their metastatic disease OR
	  ○ The member will be using Ibrance in **combination with fulvestrant** as subsequent therapy after disease progression on or following endocrine based therapy (e.g. anastrazole) for their recurrent disease OR
	  ○ The member will be using Ibrance in **combination with fulvestrant** as subsequent therapy after disease progression or following endocrine based therapy (e.g. anastrazole) for their metastatic disease.

Dependent on Patient Characteristic and Treatment Choice	• Required Medical Information: **Advanced, Recurrent, or Metastatic** Breast Cancer:
	  ○ Disease is HR-positive and HER2-negative.
	• One of the following:
	  ○ Used in **combination with an aromatase inhibitor** (e.g., anastrozole, letrozole, exemestane), OR
	  ○ Used in **combination with** fulvestrant OR
	  ○ Used as **monotherapy and disease has progressed** following endocrine therapy and patient has already received at least one prior chemotherapy regimen.
	• Required Medical Information: Early Breast Cancer:
	  ○ Diagnosis of early breast cancer at high risk of recurrence. HR-positive and HER2-negative.
	• Used in **combination with one of the following endocrine therapies**:
	  ○ tamoxifen or
	  ○ aromatase inhibitor (e.g., anastrozole, letrozole, exemestane).

Note: HR: Hormone Receptor; HER2: Human Epidermal Growth Factor Receptor 2

Figure 2: Example of Embedded Step Edit Dependent on Beneficiary Characteristic and Treatment Choice for Verzenio Prescribed as Monotherapy:

PA criteria: **Diagnosis of advanced, recurrent, or metastatic breast cancer.** Disease is HR-positive and HER2-negative and one of the following:

A.) Used in combination with an aromatase inhibitor (e.g., anastrozole, letrozole, exemestane)

B.) Used in combination with fulvestrant

C.) **Used as monotherapy and disease has progressed following endocrine therapy and beneficiary has already received at least one prior chemotherapy regimen.**

Note: HR: Hormone Receptor; HER2: Human Epidermal Growth Factor Receptor 2

Embedded Prior Authorization Requirements

Step edits embedded within PA criteria were present 23% of the time for Ibrance, 22% for Kisqali and Kisqali Femara co-pack, and 88% of the time for Verzenio (Table 2). For Ibrance, the step therapy required was dependent on treatment type, and for Kisqali, Kisqali Femara co-pack, and Verzenio it was dependent on both beneficiary characteristics and treatment type.

When step edits were required, beneficiaries were most often required to step through non-CDK 4/6 inhibitor therapy, including endocrine therapies or chemotherapy. Step edits through another CDK 4/6 inhibitor were not common, only occurring 1% of the time for Kisqali and Kisqali Femara co-pack. In these instances, plans required beneficiaries to step through either Ibrance or Verzenio.

In most cases, the ST provisions embedded in PA criteria for these Part D restrictions are similar to the prescribing information approved by the Food and Drug Administration (FDA). For example, all CDK 4/6 inhibitors are indicated to be used in combination with an aromatase inhibitor in men or in postmenopausal women, and with fulvestrant for patients with disease progression following endocrine therapy.[19,20,21] There were also cases where the ST provisions required a beneficiary to step through a preferred product, diverging from the FDA prescribing information, 1% of the time for Kisqali and Kisqali Femara co-pack.

Table 2: PA Results for Selected Drugs and Associated Treatments Across Selected Plans

Drug Name	No PA	PA						
		PA with no embedded ST	PA with ST					
			PA with ST through Other Drugs (e.g., endocrine therapy, chemotherapy)			PA with ST through a CDK 4/6 Inhibitor		
			ST Dependent on Beneficiary Characteristic	ST Dependent on Treatment Regimen	ST Dependent on both Beneficiary Characteristic and Treatment	ST Dependent on Beneficiary Characteristic	ST Dependent on Treatment Regimen	ST Dependent on both Beneficiary Characteristic and Treatment
Ibrance	3%	73%	0%	23%	0%	0%	0%	0%
Kisqali	4%	74%	0%	0%	22%	1%	0%	0%
Kisqali Femara	4%	96%	0%	0%	0%	1%	0%	0%
Verzenio	4%	8%	1%	0%	88%	0%	0%	0%

N=33,449,735 plan lives

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Conclusion

Part D plans have limited tools to mitigate increases in spending, including formulary coverage and UM, which both PDPs and MA-PDs employ to control prescription drug costs. To ensure appropriate beneficiary access to drugs, CMS reviews formularies for coverage, tier placement, and UM and requires all drugs in six protected classes to be covered by Part D plans so that beneficiaries with certain conditions can access the drugs required for their treatment.

[19] Ibrance [package insert]. New York, NY: Pfizer; 2023.
[20] Kisqali [package insert]. East Hanover, NJ: Novartis; 2023.
[21] Verzenio [package insert]. Indianapolis, IN: Lilly USA; 2023.

This analysis highlights that within the protected class of oncology, beneficiaries may face "embedded" ST in their plans' PA requirements, in this case, to access a CK4/6 inhibitor drug to treat breast cancer. Embedded steps may obscure CMS's formulary review process and pose additional barriers for patient access. CMS has methods to measure beneficiary access challenges stemming from UM or other benefit design features, through rejected claims and appeals, and should use these tools more often.

As the IRA provisions for redesigning the Part D benefit shifts liabilities in the catastrophic phase onto plans, correcting the risk adjustment model is increasingly important to avoid underpayment to plans for particular conditions. Increased plan liability and inaccurate risk adjusted payments may cause formulary design changes or encourage the use of UM that may impact access to care for patients. Additionally, with the selection of an oncology drug for 2026 IRA Medicare price negotiation, the UM dynamics within oncology may shift. These dynamics warrant attention from CMS and other stakeholders to ensure that patient access standards are upheld across plan types and therapeutic areas.

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