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PSYCHOMETRIC VALIDATON OF A QUALITY OF LIFE SCALE IN PATIENTS WITH LOW-GRADE INTERMEDIATE-RISK NON-MUSCLE INVASIVE BLADDER CANCER

Charles C. Peyton[1]*, Rahul Dhanda[2], Tom Burke[3,4], Maria Jose Aragon Aragon[3], Victoria Tsurutis[2], Nikky Ugwuoke[2], Mark Black[2], Brent Burger[2], Veronica Lee[2], Angela B. Smith[5], Michael J. Louie[2], Angela M. Stover[5]

[1]Urologic Oncology, University of Alabama at Birmingham, Birmingham, AL, USA; [2]Urogen Pharma, Princeton, NJ, USA; [3]Prime HCD, London, UK; [4]Faculty of Health and Social Care, University of Chester, Chester, UK; [5]Department of Urology and Department of Health Policy and Management, University of North Carolina at Chapel Hill, Chapel Hill, NC, USA
*Presenting author.

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INTRODUCTION

- Symptom burden and health-related quality of life (HRQoL) are critical concerns for patients with non-muscle invasive bladder cancer (NMIBC) and their healthcare providers
- The European Organization for Research and Treatment of Cancer (EORTC) Quality of Life Questionnaire (QLQ)-NMIBC24 is a questionnaire that is used to evaluate the quality of life in patients with NMIBC[1]. It has 24 items across the following 11 domains:
  - 2 functional scales/items (sexual function and sexual enjoyment)
  - 9 symptom scales/items (urinary symptoms, malaise, intravesical treatment issues, future worries, bloating and flatulence, male sexual problems, sexual intimacy, risk of contaminating a partner, and female sexual problems)
  - A high score for a functional scale represents a high/healthy level of functioning, but a high score for a symptom scale/item represents a high level of symptomatology/problems
- The instrument has not been evaluated in patients with low-grade (LG) intermediate-risk (IR) NMIBC previously
- This study evaluated and validated the psychometric properties of the QLQ-NMIBC24 and reports new suggested, between-group estimates for the minimal clinically important difference (MCID) for patients with LG-IR-NMIBC participating in a global clinical trial setting

METHODS

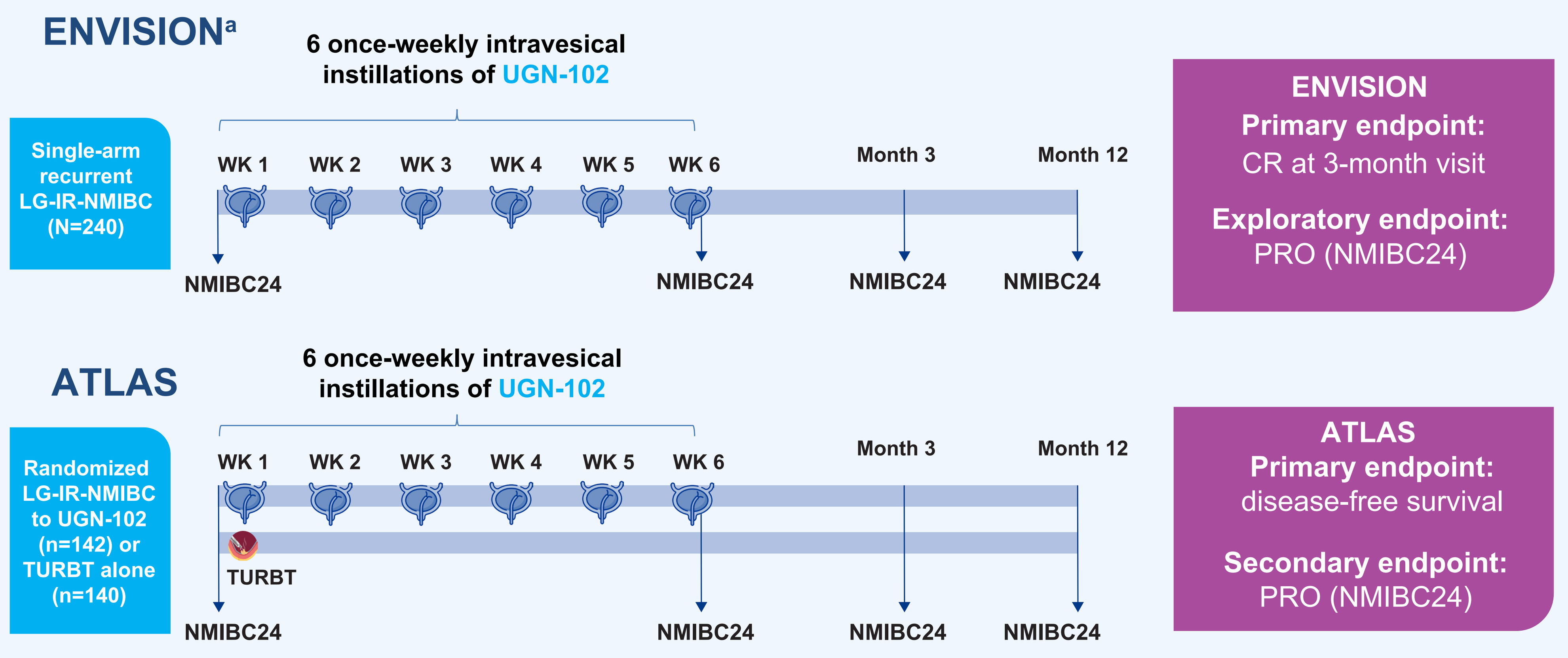
Clinical Trial Data

- Data collected in the ATLAS (NCT04688931)[2] and ENVISION (NCT05243550)[3] studies **(Figure 1)** were used to determine the psychometric properties of QLQ-NMIBC24
- ENVISION was a multinational, open-label, single-arm phase 3 trial evaluating the efficacy and safety of **UGN-102** therapy in patients with recurrent LG-IR-NMIBC
  - 240 patients received ≥1 dose of **UGN-102**, and 228 (95.0%) received all 6 weekly doses
- ATLAS was a phase 3, open-label, randomized controlled trial that assessed the efficacy and safety of **UGN-102** with or without transurethral resection of bladder tumor (TURBT) versus TURBT alone in patients with de novo or recurrent LG-IR-NMIBC
  - Patients were randomized to receive 6 once-weekly intravesical instillations of **UGN-102** (n=142) or TURBT alone (n=140)

QLQ-NMIBC24 Psychometric Evaluation and Validation

- Within the validation exercises, the test–retest reliability was performed at Week 6 and Month 3 using intraclass correlation coefficients, while internal reliability compared items using Cronbach's alpha
- For known group validity, comparisons were made using a t-test and Glass's delta standard deviations were calculated. The EORTC 30-item core quality of life questionnaire (QLQ-C30) was used to assess known-group validity using two groups, physical function scale <90 and ≥90
- Sensitivity to change was determined using analysis of covariance, where the change from baseline of the NMIBC24 domain was the dependent variable, the clinical response at Month 3 was the independent variable, and the baseline score of the NMIBC24 domain was used as the continuous variable
- Distribution-based methods were used to assess the between-group MCID. Anchor-based methods to assess MCID could not be used because anchor-based correlations were insufficient

Figure 1. ENVISION and ATLAS Study Designs


ENVISION[a]
6 once-weekly intravesical instillations of UGN-102
Single-arm recurrent LG-IR-NMIBC (N=240)
WK 1
WK 2
WK 3
WK 4
WK 5
WK 6
Month 3
Month 12
NMIBC24
NMIBC24
NMIBC24
NMIBC24
ENVISION
Primary endpoint:
CR at 3-month visit
Exploratory endpoint:
PRO (NMIBC24)
ATLAS
6 once-weekly intravesical instillations of UGN-102
Randomized LG-IR-NMIBC to UGN-102 (n=142) or TURBT alone (n=140)
WK 1
WK 2
WK 3
WK 4
WK 5
WK 6
Month 3
Month 12
TURBT
NMIBC24
NMIBC24
NMIBC24
NMIBC24
ATLAS
Primary endpoint:
disease-free survival
Secondary endpoint:
PRO (NMIBC24)

[a]The ENVISION study is ongoing and will continue for up to 63 months. The study design figure is simplified and indicates key timepoints for the presented research.
CR, complete response; LG-IR-NMIBC, low-grade intermediate-risk non-muscle invasive bladder cancer; NMIBC24, 24-item non-muscle invasive bladder cancer questionnaire; PRO, patient-reported outcomes; TURBT, transurethral resection of bladder tumor; WK, week.

RESULTS

- **Table 1** summarizes the demographics and baseline characteristics of patients enrolled in the ENVISION and ATLAS studies
- Almost all patients in ENVISION had recurrent LG-NMIBC episodes, while 38–46% of patients in ATLAS had recurrent LG-NMIBC episodes

Table 1. Baseline Demographics and Disease Characteristics

Characteristic	ENVISION	ATLAS	
	ITT Population (N=240)	UGN-102 ± TURBT ITT Population (n=142)	TURBT Alone (n=140)
Age, median (range), years	70.0 (30–92)	68 (23–85)	67 (29–88)
Sex, male, n (%)	147 (61)	105 (74)	93 (66)
Any Prior LG-NMIBC Episode, n (%)	232 (97)[a]	54 (38)	65 (46)
Prior LG-NMIBC Episode Within 1 Year of Current Diagnosis, n (%)	121 (50)	41 (29)	40 (29)
Prior TURBT, n (%)	228 (95)[a]	52 (37)	64 (46)

[a]History of LG-NMIBC requiring treatment with TURBT was an inclusion criterion for the study. A protocol deviation was recorded for patients who enrolled in the study who did not meet that criterion.
ITT, intent-to-treat; LG-NMIBC, low-grade non-muscle invasive bladder cancer; TURBT, transurethral resection of bladder tumor. Data cutoff: April 4, 2024.

- Cronbach's alpha values for the domains with more than 2 items are shown in **Table 2**
  - Except for domain malaise, values were higher than 0.75, indicating strong internal consistency
  - Interclass correlation coefficient values showed moderate-to-high test–retest reliability for the majority of domains at Week 6 and Month 3
- Known-group validity
  - Patients with high scores (>90) on the Physical function scale of the QLQ-C30 reported fewer symptoms with QLQ-NMIBC24

Table 2. Determination of Between-Group MCID

NMIBC24 domain	Number of Items in Domain	Cronbach's Alpha	MCID Between-Group Range Week 6	MCID Between-Group Range Month 3
Urinary Symptoms	7	0.8633	6.57–7.17	6.34–7.17
Malaise	2	0.3405	4.37–5.13	4.37–6.38
Intravesical Treatment Issues	1	N/A	9.38–13.85	9.38–12.55
Future Worries	4	0.8978	11.08–12.2	11.08–14.4
Bloating and Flatulence	2	0.7764	7.59–11.27	7.59–9.48
Male Sexual Problems	2	0.7844	15.3–16.29	16.29–19.84
Sexual Intimacy	1	N/A	8.69–10.48	8.69–18.43
Risk of Contaminating Partner	1	N/A	13.66–16.99	13.66–14.92
Female Sexual Problems	1	N/A	12.87–12.87	12.87–15.97
Sexual Function	2	0.8580	11.29–12.43	10.61–12.43
Sexual Enjoyment	1	N/A	11.64–17.47	11.64–12.52

MCID, minimal clinically important difference; N/A, not applicable; NMIBC24, 24-item non-muscle invasive bladder cancer questionnaire.

- Sensitivity to change, assessed from baseline to Week 6 and baseline to Month 3 showed that only urinary symptoms, sexual intimacy, and malaise were sensitive
- MCID values are shown in **Table 2**
  - At Week 6, between-group estimates of MCID ranged from 4.37 to 17.47
  - At Month 3, between-group estimates of MCID ranged from 4.37 to 19.84

CONCLUSIONS

- These results are consistent with previous reports[1] and support the utilization of EORTC QLQ-NMIBC24 to assess HRQoL in individuals with LG-IR-NMIBC
- Furthermore, the between-group, ranged, MCID estimates reported in these analyses could aid in the clinical interpretation of HRQoL in patients with LG-IR-NMIBC, and enable clinicians to evaluate the impact of different treatment interventions on patients with LG-IR-NMIBC

Limitations

- The analyses were limited by the number of patients included in the studies, and the Patient Global Impression of Change (PGIC) data, usually used for MCID estimates, were not collected
- Correlations between the QLQ-C30 and QLQ-NMIBC24 were insufficient, which meant distribution-based methods were used to calculate the MCID estimates

References
1. Blazeby JM, et al. Eur Urol. 2014;66:1148-56.
2. Prasad SM, et al. J Urol. 2023;210:619-29.
3. Prasad SM, et al. J Urol. 2025;213:205-16.

Disclaimer
UGN-102 is an investigational product.
The safety and efficacy of UGN-102 has not been established.

Disclosures
CCP: UroGen Pharma – consultant; UroGen Pharma, Janssen – scientific study; RD, VT, NU, MB, BB, VL, and MJL: UroGen Pharma – employee; TB and MJAA: Prime HCD – employee; ABS: Merck-RTI, Genentech, ARHQ, PCORI, NIH, and UroGen Pharma – grant funding; AMS: UroGen Pharma and Pfizer – research funding via institution; UroGen Pharma – one-time consulting fee ($2k) to present findings at UroGen Pharma in June 2024.

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